

CADTH RAPID RESPONSE REPORT: REFERENCE LIST

Nusinersen for Adults with Spinal Muscular Atrophy: Clinical Effectiveness

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Authors: Christopher Freige, Suzanne McCormack

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Research Question

What is the clinical effectiveness of nusinersen for adults with spinal muscular atrophy?

Key Findings

Two non-randomized studies regarding the clinical effectiveness of nusinersen for adults with spinal muscular atrophy were identified.

Methods

A limited literature search was conducted by an information specialist on key resources including Medline, Embase, the Cochrane Library, the University of York Centre for Reviews and Dissemination (CRD) databases, the websites of Canadian and major international health technology agencies, as well as a focused Internet search. The search strategy was comprised of both controlled vocabulary, such as the National Library of Medicine's MeSH (Medical Subject Headings), and keywords. The main search concepts were nusinersen or Spinraza. No filters were applied to limit the retrieval by study type. Where possible, retrieval was limited to the human population. The search was also limited to English language documents published between January 1, 2015 and January 16, 2020. Internet links were provided, where available.

Selection Criteria

One reviewer screened citations and selected studies based on the inclusion criteria presented in Table 1.

Table 1: Selection Criteria

Population	Adults with any form of spinal muscular atrophy (18 years or older)
Intervention	Nusinersen (antisense oligonucleotide [ASO])
Comparators	Alternative medications or pharmacological interventions (e.g., onasemnogene abeparvovec) Placebo No treatment
Outcomes	Clinical effectiveness: benefits/harms, hospital admissions, mortality, gross motor function (e.g., hammersmith score), mobility, quality of life, safety, adverse events
Study Designs	Health technology assessments, systematic reviews, randomized controlled trials, non-randomized studies

Results

Rapid Response 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 and non-randomized studies.

Two non-randomized studies^{1,2} regarding the clinical effectiveness of nusinersen for adults with spinal muscular atrophy were identified. No relevant health technology assessments, systematic reviews, or randomized controlled trials were identified.

Additional references of potential interest are provided in the appendix.

Health Technology Assessments

No literature identified.

Systematic Reviews and Meta-analyses

No literature identified.

Randomized Controlled Trials

No literature identified.

Non-Randomized Studies

1. Walter MC, Wenninger S, Thiele S, et al. Safety and Treatment Effects of Nusinersen in Longstanding Adult 5q-SMA Type 3 - A Prospective Observational Study. *J Neuromuscul Dis.* 2019;6(4):453-465.
[PubMed: PM31594243](#)
2. Stolte B, Totzeck A, Kizina K, et al. Feasibility and safety of intrathecal treatment with nusinersen in adult patients with spinal muscular atrophy. *Ther Adv Neurol Disord.* 2018;11:1756286418803246.
[PubMed: PM30305849](#)

Appendix — Further Information

Previous CADTH Reports

3. CADTH Common Drug Review. Nusinersen (Spinraza). Ottawa (ON): CADTH. 2019: <https://www.cadth.ca/nusinersen>
4. CADTH Common Drug Review. Nusinersen (Spinraza), resubmission. Ottawa (ON): CADTH. 2019: <https://www.cadth.ca/nusinersen-0>

Systematic Reviews and Meta-analyses

Population Not Specified

5. Claborn MK, Stevens DL, Walker CK, Gildon BL. Nusinersen: A Treatment for Spinal Muscular Atrophy. *Ann Pharmacother*. 2019 01;53(1):61-69. [PubMed: PM30008228](#)
6. Meylemans A, De Bleecker J. Current evidence for treatment with nusinersen for spinal muscular atrophy: a systematic review. *Acta Neurol Belg*. 2019 Dec;119(4):523-533. [PubMed: PM31388901](#)

Non-Randomized Studies

Mixed Population

7. Veerapandiyam A, Eichinger K, Guntrum D, et al. Nusinersen for older patients with spinal muscular atrophy: A real-world clinical setting experience. *Muscle Nerve*. 2019 Nov 26;26:26. [PubMed: PM31773738](#)
8. Pane M, Palermo C, Messina S, et al. Nusinersen in type 1 SMA infants, children and young adults: Preliminary results on motor function. *Neuromuscul Disord*. 2018 07;28(7):582-585. [PubMed: PM29960818](#)

Upcoming Trial

9. Washington University School of Medicine NCT03709784: Spinraza in Adult Spinal Muscular Atrophy (SAS). ClinicalTrials.gov. Bethesda (MD): U.S. National Library of Medicine; 2018: <https://clinicaltrials.gov/ct2/show/NCT03709784>

Conference Abstracts

10. Caumo L, Bozzoni V, Bello L, et al. Longitudinal functional changes in a cohort of adult nusinersen-treated spinal muscular atrophy patients at the Padova Neuromuscular Center. *Acta Myol*. 2019;38 (2):128. https://www.actamyologica.it/wp-content/uploads/2019/05/04_Proceedings.pdf
11. Jing Yeo CJ, Simeone S, Zhang RZ, et al. Outcome measures for nusinersen efficacy in adults with spinal muscular atrophy. *Neurology*. 2019;92(15 Supplement 1). https://n.neurology.org/content/92/15_Supplement/S5.008