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CADTH RAPID RESPONSE REPORT: REFERENCE LIST

Enzyme Replacement Therapy and Eliglustat for Gaucher Disease Type 1: Guidelines

Service Line:	Rapid Response Service
Version:	1.0
Publication Date:	September 20, 2019
Report Length:	5 Pages

CADTH

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Cite As: *Enzyme Replacement Therapy and Eliglustat for Gaucher Disease Type 1: Guidelines*. Ottawa: CADTH; 2019 Sept. (CADTH rapid response report: reference list).

About CADTH: CADTH is an independent, not-for-profit organization responsible for providing Canada's health care decision-makers with objective evidence to help make informed decisions about the optimal use of drugs, medical devices, diagnostics, and procedures in our health care system.

Funding: CADTH receives funding from Canada's federal, provincial, and territorial governments, with the exception of Quebec.

Questions or requests for information about this report can be directed to **requests@cadth.ca**

Research Question

What are the evidence-based guidelines for prescribing enzyme replacement therapies (ERT) and eliglustat in Gaucher disease Type 1?

Key Findings

No evidence-based guidelines were identified regarding prescribing ERT and eliglustat in Gaucher disease Type 1.

Methods

A limited literature search was conducted by an information specialist on key resources including Medline, 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 Gaucher Disease and enzyme replacement therapy or eliglustat. No filters were applied to limit retrieval by publication type. Where possible, retrieval was limited to the human population. The search was also limited to English language documents published between January 1, 2014 and September 16, 2019. 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	Patients of all ages with Gaucher disease Type 1
Interventions	Enzyme replacement therapies (ERT): imiglucerase, taliglucerase, velaglucerase Eliglustat
Comparator	Not applicable
Outcomes	Guidelines
Study Designs	Evidence-based guidelines

Results

Rapid Response reports are organized so that the higher quality evidence is presented first. Normally, health technology assessment reports, systematic reviews, and meta-analyses are presented first; however, in reports where guidelines are primarily sought, the aforementioned evidence types are presented in the appendix.

No evidence-based guidelines were identified regarding prescribing ERT therapies and eliglustat in Gaucher disease Type 1.

References of potential interest are provided in the appendix.

Guidelines and Recommendations

No literature identified.

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Appendix — Further Information

Previous CADTH Reports

1. Enzyme replacement therapy for the treatment of Gaucher disease type 1: clinical effectiveness. (*CADTH Rapid response report: summary of abstracts*). Ottawa (ON): CADTH; 2017: https://www.cadth.ca/enzyme-replacement-therapy-treatment-gaucher-disease-type-1-clinical-effectiveness-0. Accessed 2019 Sep 19.

2. Eliglustat (Cerdelga). (Sanofi Genzyme). Indication: Gaucher disease type 1. (*CADTH Common Drug Review*). Ottawa (ON): CADTH; 2016: https://www.cadth.ca/eliglustat. Accessed 2019 Sep 19.

3. Taliglucerase alfa (Elelyso). (Pfizer Canada Inc.). Indication: Gaucher disease. (*CADTH Common Drug Review*). Ottawa (ON): CADTH; 2014: https://www.cadth.ca/taliglucerase-alfa-4. Accessed 2019 Sep 19.

Health Technology Assessments

4. All Wales Medicines Strategy Group (AWMSG). Velaglucerase alfa. (*AWMSG Secretariat Assessment Report Advice No. 1214*). Penarth: All Wales Therapeutics and Toxicology Centre (AWTTC), secretariat of the All Wales Medicines Strategy Group (AWMSG); 2014: http://www.awmsg.org/awmsgonline/app/appraisalinfo/571. Accessed 2019 Sep 19.

Systematic Reviews and Meta-analyses

5. Nabizadeh A, Amani B, Kadivar M, et al. The clinical efficacy of imiglucerase versus eliglustat in patients with Gaucher's disease type 1: a systematic review. *J Res Pharm Pract*. 2018 Oct-Dec;7(4):171-177. PubMed: PM30622983

6. Shemesh E, Deroma L, Bembi B, et al. Enzyme replacement and substrate reduction therapy for Gaucher disease. *Cochrane Database Syst Rev*. 2015 Mar 27(3):CD010324. PubMed: PM25812601

7. Smid BE, Hollak CE. A systematic review on effectiveness and safety of eliglustat for type 1 Gaucher disease. *Expert Opin Orphan Drugs*. 2014; 2(5): 523-529.

Clinical Practice Guidelines and Recommendations

Unclear Methodology

8. Puri RD, Kapoor S, Kishnani PS, et al. Diagnosis and management of Gaucher disease in India - consensus guidelines of the Gaucher Disease Task Force of the Society for Indian Academy of Medical Genetics and the Indian Academy of Pediatrics. *Indian Pediatr*. 2018 02 15;55(2):143-153. PubMed: PM29503270

9. Belmatoug N, Di Rocco M, Fraga C, et al. Management and monitoring recommendations for the use of eliglustat in adults with type 1 Gaucher disease in Europe. *Eur J Intern Med.* 2017 Jan;37:25-32.
PubMed: PM27522145

10. Balwani M, Burrow TA, Charrow J, et al. Recommendations for the use of eliglustat in the treatment of adults with Gaucher disease type 1 in the United States. *Mol Genet Metab*. 2016 Feb;117(2):95-103.
PubMed: PM26387627

Review Articles

11. Gary SE, Ryan E, Steward AM, Sidransky E. Recent advances in the diagnosis and management of Gaucher disease. *Expert Rev Endocrinol Metab*. 2018 03;13(2):107-118.
PubMed: PM30058864

12. Gupta P, Pastores G. Pharmacological treatment of pediatric Gaucher disease. *Expert Rev Clin Pharmacol*. 2018 Dec;11(12):1183-1194.
PubMed: PM30444430

13. Rosenbaum H. Management of women with Gaucher disease in the reproductive age. *Thromb Res*. 2015 Feb;135 Suppl 1:S49-51.
PubMed: PM25903536