CADTH Reference List

Somatropin for Idiopathic Short Stature

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Summary of Abstracts

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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.

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Key Message

We identified 1 evidence-based guideline about the use of growth hormone therapy for children with idiopathic short stature.

Research Question

What are the evidence-based guidelines regarding the use of growth hormone therapy for children with idiopathic short stature?

Methods

Literature Search Methods

An information specialist conducted a literature search on key resources including MEDLINE, Embase, the Cochrane Database of Systematic Reviews, the International HTA Database, the websites of Canadian and major international health technology agencies, as well as a focused internet search. The search approach was customized to retrieve a limited set of results, balancing comprehensiveness with relevancy. The search strategy comprised both controlled vocabulary, such as the National Library of Medicine's MeSH (Medical Subject Headings), and keywords. Search concepts were developed based on the elements of the research questions and selection criteria. The main search concepts were human growth hormone and idiopathic short stature. CADTH-developed search filters were applied to limit retrieval to guidelines. The search was completed on June 14, 2023, and limited to English-language documents published since January 1, 2013.

Selection Criteria and Summary Methods

One reviewer screened literature search results (titles and abstracts) and selected publications according to the inclusion criteria presented in Table 1. Full texts of study publications were not reviewed. The Overall Summary of Findings was based on information available in the abstracts of selected publications. Open access full-text versions of evidence-based guidelines were reviewed when available and relevant recommendations were summarized.

Table 1: Selection Criteria

Criteria	Description
Population	Children (< 18 years old) with idiopathic short stature
Intervention	All somatropin products
Comparator	Not applicable
Outcomes	Recommendations regarding best practices (e.g., dose and timing of treatment, duration of treatment, laboratory cut-offs for eligibility, monitoring treatment response)
Study designs	Evidence-based guidelines

Results

One evidence-based guideline was identified regarding the use of growth hormone (GH) therapy for children with idiopathic short stature (ISS).[1]

One guidance recommendation that did not meet the inclusion criteria due to unclear methodology is summarized in Appendix 1. Additional references of potential interest that did not meet the inclusion criteria are provided in Appendix 2.

Overall Summary of Findings

The identified guideline provides recommendations regarding GH treatment for children and adolescents with ISS, specifically on the treatment decision-making approach (recommendations 6.1), routine use (recommendation 6.1), treatment response monitoring (recommendation 6.2), and initial dosage (recommendations 6.3).[1] Refer to Table 2 for a detailed summary of relevant recommendations.

Table 2: Summary of Recommendations in Included Guideline

Summary of recommendations	Quality of evidence and strength of recommendations
Grimberg et al. (2017) [1]	
"6.1. In the US, for children who meet FDA criteria, we suggest a shared decision-making approach to pursuing GH treatment for a child with ISS. The decision can be made on a case-by-case basis after assessment of physical and psychological burdens and discussion of risks and benefits. We recommend against the routine use of GH in every child with HtSDS ≤ −2.25." (p. 364, 381)	**Quality of evidence:** Moderate **Strength of recommendation**: Conditional
"6.2. We suggest a follow-up assessment of benefit in HtSDS and psychosocial impact 12 months after GH initiation and dose optimization." (p. 365, 381)	**Quality of evidence**: Low **Strength of recommendation**: Conditional
"6.3. Because there is overlap in response between dosing groups, we suggest initiating GH at a dose of 0.24 mg/kg/week, with some patients requiring up to 0.47 mg/kg/week." (p. 365, 382)	**Quality of evidence**: Low **Strength of recommendation**: Conditional

HtSDS = height standard deviation score; ISS = idiopathic short stature; SDS = standard deviation score.

Reference

Guidelines and Recommendations

1. Grimberg A, DiVall SA, Polychronakos C, et al. Guidelines for Growth Hormone and Insulin-Like Growth Factor-I Treatment in Children and Adolescents: Growth Hormone Deficiency, Idiopathic Short Stature, and Primary Insulin-Like Growth Factor-I Deficiency. *Hormone Research in Paediatrics*. 2017 01 Jan;86(6):361-397. PubMed

Refer to: Recommendations 6.1 to 6.3 (p.364 to 365, 381 to 383)

Appendix 1: Summary of Identified Guideline

The guidance from Ministry of Health Singapore did not meet the methodological criteria to be considered evidence-based, and therefore was not eligible for inclusion in the main body of this report. However, it provides 1 recommendation against the use of somatropin in children with ISS.

Guidelines and Recommendations

Unclear Methodology

Ministry of Health Singapore. Somatropin for the treatment of growth failure in children Technology Guidance from the MOH Drug Advisory Committee; 2017. ACE | Somatropin for the treatment of growth failure in children (ace-hta.gov.sg) Accessed 2023 June 15.

Appendix 2: References of Potential Interest

Review Articles

Review of Guidelines and Recommendations

Al Herbish AS, Almutair A, Bin Abbas B, et al. Diagnosis and management of growth disorders in Gulf Cooperation Council (GCC) countries: Current procedures and key recommendations for best practice. *International Journal of Pediatrics and Adolescent Medicine*. 2016 01 Sep;3(3):91-102. PubMed

Additional References

Authorization Criteria

Florida Agency for Health Care Administration. Growth hormone treatment in children and adults; 2022. https://ahca.myflorida.com/content/download/8665/file/Human_Growth_Hormone_Criteria.pdf Accessed 2023 June 15.
Refer to: Review criteria for children, p. 1; Discontinuation of growth hormone therapy in children, p. 4; Criteria for continuation of growth hormone therapy in children, p. 4