TITLE: Human Growth Hormone Treatment for Prader-Willi Syndrome in Adolescent and Adult Patients: Clinical Evidence, Safety and Guidelines

DATE: 27 August 2015

RESEARCH QUESTIONS

1. What is the clinical effectiveness of human growth hormone treatment for Prader-Willi syndrome in adolescent and adult patients?

2. What are the evidence-based guidelines for the use of human growth hormone treatment for Prader-Willi syndrome in adolescent and adult patients?

KEY FINDINGS

Two systematic reviews, one randomized controlled trial, 13 non-randomized studies, and one evidence based guideline were identified regarding the clinical effectiveness and use of human growth hormone treatment for Prader-Willi syndrome in adolescent and adult patients.

METHODS

A limited literature search was conducted on key resources including PubMed, The Cochrane Library, University of York Centre for Reviews and Dissemination (CRD), Medline, Embase, Canadian and major international health technology agencies, as well as a focused Internet search. No methodological filters were applied. Where possible, retrieval was limited to the human population. The search was also limited to English language documents published between January 1, 2012 and August 21, 2015. Internet links were provided, where available.

SELECTION CRITERIA

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

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<table>
<thead>
<tr>
<th>Population</th>
<th>Adult or adolescent patients with Prader-Willi syndrome</th>
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<tbody>
<tr>
<td>Intervention</td>
<td>Human growth hormone</td>
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<tr>
<td>Comparator</td>
<td>Q1: Any</td>
</tr>
<tr>
<td></td>
<td>Q2: No comparator</td>
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<tr>
<td>Outcomes</td>
<td>Q1: Clinical effectiveness (e.g., improvement in symptoms [symptoms include, but are not limited to: infertility, hypogonadism, obesity, hypotonia, learning disabilities, increased diabetes risk, extreme flexibility]); Safety (e.g., risks associated with treatment, contraindications)</td>
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<td>Q2: Evidence-based guidelines</td>
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<td>Study Designs</td>
<td>Health technology assessments, systematic reviews, meta-analyses, randomized controlled trials, non-randomized studies, evidence-based guidelines</td>
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**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, non-randomized studies, and evidence-based guidelines.

Two systematic reviews (one with meta-analysis), one randomized controlled trial, 13 non-randomized studies, and one evidence based guideline were identified regarding the clinical effectiveness and use of human growth hormone treatment for Prader-Willi syndrome in adolescent and adult patients. No relevant health technology assessments were identified.

Additional references of potential interest are provided in the appendix.

**Health Technology Assessments**
No literature identified.

**Systematic Reviews and Meta-analyses**


**Randomized Controlled Trials**

Non-Randomized Studies


Guidelines and Recommendations

See: Table 1. Summary of Clinical Care Guidelines for rhGH Therapy in PWS, page E1075
APPENDIX – FURTHER INFORMATION:

Non-Randomized Studies

Unclear Age


Ongoing Studies


Review Articles


