TITLE: Tiopronin for Cystinuria in Pediatric Patients: Clinical Effectiveness, Cost-Effectiveness, and Guidelines

DATE: 24 January 2017

RESEARCH QUESTIONS

1. What is the clinical effectiveness of tiopronin for the prevention of kidney stone formation in pediatric patients diagnosed with cystinuria?

2. What is the cost-effectiveness of tiopronin for the prevention of kidney stone formation in pediatric patients diagnosed with cystinuria?

3. What are the evidence-based guidelines regarding the use of tiopronin in pediatric patients diagnosed with cystinuria?

KEY FINDINGS

No relevant literature was identified regarding the clinical or cost-effectiveness of tiopronin for the prevention of kidney stone formation in pediatric patients diagnosed with cystinuria. In addition, no evidence-based guidelines were identified regarding the use of tiopronin in pediatric patients diagnosed with cystinuria.

METHODS

A limited literature search was conducted on key resources including PubMed, The Cochrane Library, University of York Centre for Reviews and Dissemination (CRD) databases, Canadian and major international health technology agencies, as well as a focused Internet search. Methodological filters were applied to limit retrieval to health technology assessments, systematic reviews, meta-analyses, randomized controlled trials and economic studies. Where possible, retrieval was limited to the human population. The search was also limited to English language documents published between January 1, 2009 and December 18, 2016. 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>
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<th>Table 1: Selection Criteria</th>
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<td><strong>Population</strong></td>
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<td><strong>Intervention</strong></td>
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<td><strong>Comparator</strong></td>
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| **Outcomes**                | Q1: Clinical benefits and harms (e.g., prevention of kidney stone formation, adverse events)  
|                             | Q2: Cost-effectiveness outcomes (e.g., cost per QALY or health benefit)  
|                             | Q3: Evidence-based guidelines |
| **Study Designs**           | Health technology assessments, systematic reviews, meta-analyses, randomized controlled trials, non-randomized studies, economic evaluations, evidence-based guidelines |

RESULTS

No relevant health technology assessments, systematic reviews, meta-analyses, randomized controlled trials, non-randomized studies, or economic evaluations were identified regarding the clinical or cost-effectiveness of tiopronin for the prevention of kidney stone formation in pediatric patients diagnosed with cystinuria. In addition, no evidence-based guidelines were identified regarding the use of tiopronin in pediatric patients diagnosed with cystinuria.

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**

No literature identified.

**Economic Evaluations**

No literature identified.

**Guidelines and Recommendations**

No literature identified.
APPENDIX – FURTHER INFORMATION:

Non-Randomized Studies

**Pediatric Case Reports**


**No Specific Mention of Tiopronin**


**Adult Case Report – Alternate Indication**


**Conference Abstracts**

**Pediatric Case Report (Alternate Indication)**


**Mixed or Unclear Population**


Guidelines and Recommendations – Adult Population


NGC summary available from: https://guideline.gov/summaries/summary/48229
See: Recommendation 20

Clinical Practice Guidelines – Unspecified Methodology

Unsure if Specific Recommendations are for the Pediatric Population

See: Figure 6

Review Articles


PubMed: PM25383320


