Evidence Evaluation & Methods Workgroup: Developing a Decision Analysis Model

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Overview

• Limitations of evidence review
• Brief introduction to decision analysis
• Case study: Newborn screening for MCADD
• Application to hyperbilirubinemia
ACHDNC: Evidence Evaluation Methods Working Group

• Convened in April, 2011
• Charged with evaluating evidence review methods
• Considered modeling to assist in evidence synthesis and generation
• Hyperbilirubinemia case study
Decision Analysis (DA)

- Systematic approach to decision making under conditions of uncertainty
- Requires explicit consideration of each aspect of the decision problem:
  - Defining full set of alternatives
  - Choices regarding timing of implementation
  - Uncertainties involved
  - Assigning relative values to full set of possible outcomes
- Identifies alternative estimated to result in maximum benefit and uncertainty associated with that projection
Advantages of Modeling

• Can evaluate existing & un-tested alternatives
• Can simulate head-to-head comparisons
• Requires explicit definition of assumptions
• Can identify sources of uncertainty/prioritize future research
• Allows for extension of time horizons
Decision Analysis & Child Health Policy

• Decision analytic modeling approach can provide insights into comparative effectiveness and cost-effectiveness

• Especially salient for child health by providing approach for projecting long-term outcomes

• Cost-effectiveness results increasingly considered
Decision Analysis & Newborn Screening

• Incorporation of modeling into the evidence review process:
  – Simple models
  – Health outcomes
  – No cost-effectiveness analysis (yet)

• Initial goal is to project health benefits and potential harms
Case Study:
Expanded Newborn Screening for MCADD

• Expansion of newborn screening programs using MS-MS
• Incremental costs very low
• Total costs of screening and follow-up not characterized
• Higher incidence with newborn screening

Source: Prosser et al., *Pediatrics*, 2010
MCADD Example: Methods

• Develop a decision analytic model to project health benefits, health risks, and costs of expanded newborn screening
• Model inputs using primary and published data supplemented by expert opinion:
  – Event probabilities (e.g., outcomes of metabolic disease, test characteristics)
  – Costs of screening and clinical outcomes
  – Effects on health-related quality of life (HRQOL)
• Limited evidence especially for long-term outcomes
• Analysis: Projected long-term clinical outcomes and cost-effectiveness
MCADD Example: Decision Analytic Model

Newborn Screening Simulation Model

- Costs
- Probabilities
- Health State Values

Screening Outcomes
- False positives
- Follow-up required

Clinical Outcomes
- Cases
- Hospitalizations
- Deaths

Economic Outcomes
- Costs
- QALYs
MCADD Example: Decision Analytic Model

- **Costs**
- **Probabilities**
- **Health State Values**

### Outcomes
- **Screening Outcomes**
  - False positives
  - Follow-up required
- **Clinical Outcomes**
  - Cases
  - Hospitalizations
  - Deaths
- **Economic Outcomes**
  - Costs
  - QALYs
MCADD Newborn Screening Simulation Model

- Hypothetical Cohort of Newborns
  - Newborn Screening Program Submodel
  - No Newborn Screening Program
  - Lifetime MCADD Submodel with Screening
  - Lifetime MCADD Submodel with Clinical Identification
Lifetime MCADD Submodel

From Newborn Screening Program Submodel

- Normal
- Intellectual Disability
- Dead

Hospitalization
## MCADD Example: Projected Outcomes

<table>
<thead>
<tr>
<th></th>
<th>Clinical Ident.</th>
<th>Screening</th>
<th>Δ</th>
</tr>
</thead>
<tbody>
<tr>
<td>Population</td>
<td>100,000</td>
<td>100,000</td>
<td>-</td>
</tr>
<tr>
<td>Children w/MCADD</td>
<td>5.88 (0.01)</td>
<td>8.4 (0.01)</td>
<td>2.52</td>
</tr>
<tr>
<td>FP screen</td>
<td>N/A</td>
<td>20 (0.02)</td>
<td>20</td>
</tr>
<tr>
<td>Costs (lifetime)</td>
<td>$630,710</td>
<td>$1,629,482</td>
<td>$998,778</td>
</tr>
<tr>
<td>QALYs</td>
<td>2,976,780.08</td>
<td>2,976,827.03</td>
<td>46.95</td>
</tr>
<tr>
<td>C/E ratio</td>
<td></td>
<td>$21,273 (395)</td>
<td></td>
</tr>
</tbody>
</table>

Source: Prosser et al., *Pediatrics*, 2010
MCADD Example: Projected Long-term Outcomes

Source: Prosser et al., Pediatrics, 2010
MCADD Example: Policy Implications

• Model predicts short- and long-term outcomes
  – Screening test and follow-up results
  – Projected number of children with condition, cases of developmental delay, hospitalizations, deaths
  – Costs
  – Quality-adjusted life years

• Results robust to changes in underlying assumptions

• Results sensitive to test costs, but not false positive rate
Application to Hyperbilirubinemia

- Create decision analysis model
- Expert panel input
- Project short- and long-term health outcomes
# Projected Health Outcomes

<table>
<thead>
<tr>
<th>Selected Outcomes</th>
<th>Clinical Assessment</th>
<th>Universal Screening</th>
</tr>
</thead>
<tbody>
<tr>
<td>Screening outcomes</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Short-term:</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Hospitalizations</td>
<td></td>
<td></td>
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<tr>
<td>Long-term:</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Acute bilirubin encephalopathy</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Chronic bilirubin encephalopathy</td>
<td></td>
<td></td>
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<tr>
<td>Unaffected</td>
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</tbody>
</table>
Anticipated Findings

- Projected health outcomes and associated uncertainty
- Identification of key parameters
- Process for specifying assumptions on health benefits and potential risks
Moving forward

• Adding decision analysis to the evidence review can provide:
  – Approach for evidence synthesis
  – Method for specifying assumptions

• Incorporation of cost/cost-effectiveness analysis
Questions