Use of Benefit-Cost Analysis in Washington State’s Newborn Screening Policy Process

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Acknowledgments

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The findings and conclusions in this presentation have not been formally disseminated by the Centers for Disease Control and Prevention and should not be construed to represent any agency determination or policy.
US Newborn Screening Policy

- **State responsibility**
  - Each state defines by law or regulation a set of disorders for which all newborns should be screened through either laboratory analysis of dried blood spots (DBS) collected soon after birth or point-of-care testing

- **Federal role**
  - Since 2005 a federal Advisory Committee on Heritable Disorders in Newborns and Children has recommended a set of disorders for states to screen
  - Since 2010, the Secretary of Health and Human Services has defined a Recommended Uniform Screening Panel, but states decide whether and when to implement the recommendations
Washington State Process to Add Disorders to NBS Panel

1. Nomination of a proposed disorder
2. State Board of Health reviews nominations and makes decision whether to convene ad hoc Newborn Screening Advisory Committee
3. Advisory Committee reviews evidence of feasibility & benefit
4. If disorder meets those criteria, economic analysis is prepared by Department of Health staff
5. Advisory committee makes recommendation to BOH
6. Board of Health decides whether to add disorder to panel
7. Implementation by Department of Health may require Legislature to raise the NBS fee charged to hospitals
Washington State Requirement for Regulatory Benefit-Cost Analysis

- **Washington Administrative Procedure Act:**
  - Before adopting a rule…an agency shall: determine that the probable benefits of the rule are greater than its probable costs, taking into account both the qualitative and quantitative benefits and costs and the specific directives of the statute being implemented [Revised Code of Washington (RCW) 34.05.328 Section (1)(b)]
Washington State’s Use of BCA in NBS Policy

- Since 2002 WDOH has developed spreadsheet benefit-cost models prior to each NBS expansion
  - 2003 – 5 metabolic disorders, including MCAD deficiency
  - 2005 – cystic fibrosis (CF)
  - 2008 – 15 other metabolic conditions included in the federally recommended screening panel
  - 2012 – Severe combined immune deficiency (SCID)
  - All models reported net benefit

- Models prepared by WDOH economists and NBS program analysts with clinical input

Timeline for 2003 Models

- **2001** – NBS Advisory Committee requested to consider adding 9 disorders
  - Committee decides 6 disorders have sufficient evidence
  - Hearing loss economic assessment is outsourced
  - Economic models for 5 metabolic disorders set up at WDOH

- **2002** Advisory Committee recommends all 5 disorders based on estimates of net benefit
  - Cost of new MS/MS technology attributed to one disorder, MCAD deficiency

- **2003** Board of Health approves 5 disorders after economic models are revised

- Screening for 5 disorders initiated in 2004
BCA Models: Costs of screening, diagnosis and treatment with and without NBS

- **Costs to public health departments**
  - Laboratory testing
    - Staff costs
    - Equipment and reagents
    - Space and utilities
  - Short-term follow-up and tracking

- **Downstream costs**
  - Clinical follow-up from screening through diagnosis
  - Long-term management
    - Difference in cost of treatment following early diagnosis
  - Education costs (for some conditions)
BCA Models: Benefits of NBS Expansion

- **Avoided disability costs (2003 and 2008 models)**
  - Lifetime direct medical, education, and indirect productivity costs based on 2003 RTI/CDC modeling study
  - Human capital, not willingness to pay (WTP)

- **Reduction in mortality (all models)**
  - Deaths valued using VSL

- **VSL estimates**
  - 2003 models: $4.0 million, range $1-16 million
  - 2005 model: $4.0 million, no range
  - 2008 models: $4.4 million, range $1-7 million
  - 2012 model: $7.7 million, range $6.1-9.1 million
2003 Model for MCAD Deficiency

- Medium chain acyl-CoA dehydrogenase (MCAD) deficiency occurs in about 1 in 15-25,000 infants
  - Fasting can cause metabolic decompensation crises resulting in
    • Emergency hospitalizations in 70-75%
    • Can be fatal in 20% of infants without diagnosis
    • Developmental disability in 5-20% of survivors
  - Easily treated by ensuring infants do not go without eating

- Screening modeled over 10 year period
  - 827,000 infants screened at $12 each
  - 35 infants detected with MCAD deficiency
    • 7 deaths averted
    • 7 cases of developmental disability avoided
    • 27 avoided acute hospitalizations
## Modeled Benefits and Costs of MCAD Screening: Present Values over 10 Year Period

<table>
<thead>
<tr>
<th></th>
<th>Millions of dollars</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Total Cost</strong></td>
<td>9.9</td>
</tr>
<tr>
<td>Screening and follow-up</td>
<td>8.7</td>
</tr>
<tr>
<td>Hospitalizations for observation</td>
<td>0.8</td>
</tr>
<tr>
<td><strong>Total Benefit</strong></td>
<td>32.6</td>
</tr>
<tr>
<td>Mortality from metabolic crises</td>
<td>28.1</td>
</tr>
<tr>
<td>Developmental disability</td>
<td>4.2</td>
</tr>
<tr>
<td>Emergency hospitalizations</td>
<td>0.3</td>
</tr>
<tr>
<td><strong>Net Benefit</strong></td>
<td>22.7</td>
</tr>
</tbody>
</table>
Modeling the Cost of Avoided Developmental Disability from MCAD Deficiency

- 4.5 cases of intellectual disability, at $870,000 each
- 2.5 cases of cerebral palsy alone, at $800,000 each
- Distribution of per-child costs (Honeycutt et al. 2003):

<table>
<thead>
<tr>
<th>Cost type</th>
<th>Intellectual disability</th>
<th>Cerebral palsy</th>
</tr>
</thead>
<tbody>
<tr>
<td>Productivity loss – mortality</td>
<td>7%</td>
<td>13%</td>
</tr>
<tr>
<td>Productivity loss – disability</td>
<td>72%</td>
<td>69%</td>
</tr>
<tr>
<td>Special education</td>
<td>7%</td>
<td>7%</td>
</tr>
<tr>
<td>Medical costs</td>
<td>14%</td>
<td>10%</td>
</tr>
</tbody>
</table>

Retrospective Evaluation of WA MCAD Model

- Estimates of avoided mortality consistent with other evidence
  - VSL estimate consistent with contemporary estimates
  - Newer VSL estimates are substantially higher

- Developmental disability in MCAD deficiency
  - Subsequent evidence suggests occurrence about $\frac{1}{4}$ as high
  - Per-child cost estimates were crude
    - No adjustment for severity or co-occurring conditions
    - Excluded parental productivity losses,
  - Likely lower than WTP for prevention of serious illness & disability

- Estimation of net benefit was conservative
Lessons Learned from WA Experience

- **Modeling benefits of expanding NBS is challenging**
  - Requires review of epidemiologic and clinical evidence
    - Easier if systematic reviews and cost-effectiveness analyses are available
      - MCAD, CF and SCID models drew on previous studies
      - Other models were less robust due to lack of data
  - Mortality is straightforward as an endpoint
  - Developmental disability is complicated
    - Heterogeneity
    - Lack of directly relevant cost estimates
    - No estimates of WTP for nonfatal endpoints

- **BCA modeling requires resources and time**
  - WA NBS program has developed internal modeling capacity