OTA’s Newborn Screening Study: Relevance to Today’s Issues?

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OTA’s Newborn Screening Study

• Analysis in 1986-7; published Feb 1988
• Chapter 5 in “Healthy Children: Investing in the Future”
  http://www.wws.princeton.edu/~ota/ns20/alpha_f.html
• Response to Congressional Committee Request: “Tell us what preventive measures are cost-effective for infants and children.”
• OTA studied early prenatal care, newborn screening, well-child care, accidental injuries, child maltreatment
Findings on Newborn Screening

• USA and Canada are the only developed countries without a national screening program.
• Lack of a coordinated network of newborn screening services in some areas may reduce the overall effectiveness of newborn screening.
• Expanding newborn screening strategies to include additional diseases (HC, GA, MSUD) beyond PKU and CH, and/or to take second specimen would save more newborns from death and disability, but the incremental costs per case found would be high.
Limitations of OTA’s CEA

- Outcome measure outmoded (cases detected per 100,000 infants screened)
- Interpretation faulty: Cost probably NOT high if converted to healthy life-years saved
- Discount rate on future costs (7%) higher than today’s CEA standard (3%)
- Data limited on outcomes of disease and of screening.
- Screening technologies old.
Elements of Screening Intervention

• Number of samples, timing relative to birth, and location of sample collection
• Diseases to be tested for
• Screening technology(ies) to be used
• Laboratory procedures (e.g., quality assurance, GLP)
• Confirmatory procedures
• Follow-up and treatment regimens

How interventions are defined and what baseline program they are compared with influence both the findings and the usefulness of analysis.
OTA’s Strategies

(1) PKU + CH 1 SPECIMEN

(2) +2nd Spec PKU+CH All Infants
(3) +2nd Spec PKU+CH Early Discharge
(4) +2nd Spec CH All Infants
(5) +2nd Spec PKU+CH+HC All Infants
(6) + GA, MSUD on 1st Spec
(7) + GA, MSUD on 1st Spec +2nd Spec PKU+CH+HC
<table>
<thead>
<tr>
<th>Strategy</th>
<th>Net Cost cf Baseline</th>
<th>Extra Cases Found cf Baseline</th>
<th>C/E cf Baseline</th>
</tr>
</thead>
<tbody>
<tr>
<td>Strategy 2</td>
<td>942,000</td>
<td>2.0</td>
<td>471,000</td>
</tr>
<tr>
<td>Strategy 3</td>
<td>330,000</td>
<td>1.3</td>
<td>253,845</td>
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<tr>
<td>Strategy 4</td>
<td>735,000</td>
<td>1.7</td>
<td>432,353</td>
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<tr>
<td>Strategy 5</td>
<td>1,052,000</td>
<td>2.5</td>
<td>420,800</td>
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<tr>
<td>Strategy 6</td>
<td>313,000</td>
<td>1.8</td>
<td>173,889</td>
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<tr>
<td>Strategy 7</td>
<td>1,364,000</td>
<td>4.3</td>
<td>317,209</td>
</tr>
</tbody>
</table>

Cf = compared with
OTA’s Incremental Strategies

(1) PKU + CH
1 SPECIMEN

(2) +2nd Spec
PKU + CH
All Infants

(3) +2nd Spec
PKU + CH
Early Discharge

(4) +2nd Spec
CH
All Infants

(5) +2nd Spec
PKU + CH + HC
All infants

(6) + GA, MSUD
on 1st Specimen

(7) + 2nd Spec
PKU, CH & HC
All infants

Baseline
Wisconsin Study

NO Screening for MCAD

MS/MS Screening for MCAD

Baseline

Insigna, et al., J Pediatrics, 2002: 141(4), 524-531
NHS/HTA Study

Existing PKU Screening Program

PKU Screening via MS/MS Technology +MCAD

+GAI +HC +MSUD +LC FA Defects +Etc.

Baseline

Considerations for today’s CEAs

• Impact of Private Sector Labs
  – Costs and savings outside the public sector
  – Possible cost saving in capital investment in MS/MS equipment and specialized training of personnel
  – Possible loss of fees to state
  – Supplemental test panel offered for additional fee: equity issues
Current Issues, cont.

• Wider Range of Screening Outcomes
  – Provide treatment to avoid neonatal mortality or severe mental retardation
  – Offer treatment that may reduce morbidity later in life
  – Family planning purposes only
  – Research; no immediate clinical benefit to affected infants or their families
Current Issues, cont.

- Wider range of available tests; need for evaluating outcomes and effectiveness
  - Impact of screening organization:
    - Regional systems
    - Centralizing labs (public/private)
  - Effects of reducing disparities among states: reduced numbers of missed cases
  - Potential role of Federal-state partnerships in financing and guiding implementation of national goals