

Evaluating Harms of Newborn Screening Within Evidence Review

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Goal:

For each nominated condition

Support Committee assessment of net benefit

Approach:

- 1) Integrate into Evidence Review**
- 2) Utilize to model net benefit**



Manuscript:

**“Evaluating Harms in the Assessment of Net Benefits:
A Framework for Newborn Screening Condition Review”**

- 1) Aaron J. Goldenberg - Case Western
- 2) Anne Comeau - New England NBS Program
- 3) Scott D. Grosse - CDC
- 4) Susan Tanksley - Texas Dept. State Health Services
- 5) Lisa A. Prosser – U. Michigan
- 6) Jelili Ojodu - APHL
- 7) Jeffrey R. Botkin – U. Utah, SAC member
- 8) Alex R. Kemper – Duke U.
- 9) Nancy S. Green – Columbia U.

For the SACHDNC



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Process:

- 1) Evidence Review group
- 2) Input from SACHDNC leadership and membership (Dr. Botkin)
- 3) Review of methodology used by other evidence-based evaluative bodies:
 - **USPSTF**
 - **EGAPP**
 - **Others (e.g. ACIP, IOM, thought leaders)**



3 Decisions:

1) Define harms: Any adverse impact

- Adverse events, Burdens, Risks

2) Primary consideration: **Child**

- Family and social considerations: **Included**

3) Harms considered:

- ***Beyond*** those arising from standard clinical presentation and care
 - Include children deriving no clinical benefit



Harms to consider:

Screening, diagnostic evaluation, treatment

Child:

- Physical burden to infants
- Increased risk of medical treatment (e.g. earlier Rx)
- Delayed diagnosis from false negative results
- Uncertainty of clinical diagnosis or clinical spectrum
- Disparities in access

Family:

- Psychosocial and logistic burdens (e.g. false positives)



Challenges: Both **generic** and **particular** to NBS

- 1) Trials generally focus on medical benefits
- 2) Limited data on harms:
 - Less available, less apparent, short-term focus
- 3) Subject recruitment and selectivity
 - Constrained numbers and sampling (e.g. sibs, diversity)



Approach for Evidence Review:

1) Formalize review of harms (*in place*)

2) Consider impact:

- Number of children at risk
- Severity
- Likelihood
- Timing

(Opportunity costs for NSB programs –
by public health assessment)

3) Methodology: modeling, + pilot data, + research



Current status:

- Recommendations have been incorporated into methodology of the Evidence Review Workgroup

Next steps:

- Committee's final comments on the manuscript
- Submission to peer-reviewed publication

Discussion?



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