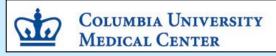
Evaluating Harms of Newborn Screening Within Evidence Review

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SACHDNC 2/13/2015



Goal:

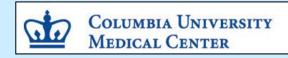
For each nominated condition

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



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) <u>Define harms</u>: 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)



<u>Challenges</u>: 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)

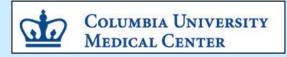
Approach for Evidence Review:

- 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



