Duke Clinical Research Institute

Assessing Public Health System Impact

Alex R. Kemper, MD, MPH, MS

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Public Health System Impact Assessment

RECAP	Public Health System Assessment Development
2012	Expert Advisory Panel (EAP) to develop Public Health Impact Decision Considerations and Assessment
2013-2014	Committee Decision Matrix revised to include Public Health Impact (Kemper et al., 2014)
2012-2013	Pilot PHSI Assessment for Condition Review of Pompe Disease: Surveys, in-depth interviews, selected state NBS programs (n=9)
2014	EAP-2 to refine PHSI Assessment procedures

Using Feasibility and Readiness in the Decision-Making Process

- **Feasibility: High/Moderate or Low**
  - established and available screening test
  - clear approach to diagnostic confirmation
  - treatment plan acceptable to clinicians and individuals and families
  - long-term follow-up plans can be established
- **Readiness: Ready, Developmental Readiness, or Unprepared**
  - **After the state makes the decision to include the condition and funding is made available**
  - **Feasibility = central issue for the Committee to recommend addition to the RUSP**
  - **Readiness assessment follows feasibility assessment**
  - **Readiness ratings:**
    - » ***Ready***:
      - Most NBS programs could implement within 1 year
    - » ***Developmental Readiness***:
      - Most NBS programs could implement within 1–3 years
    - » ***Unprepared***:
      - Most NBS programs could implement in >3 years

Kemper et al. (2014). Decision-making process for conditions nominated to the Recommended Uniform Screening Panel: Statement of the US DHHS Secretary's Advisory Committee on Heritable Disorders in Newborns and Children. Gen Med, 16, 183–187.

SACHDNC Decision Matrix

PHSI

NET BENEFIT		FEASIBILITY	READINESS		
			Ready	*Developmental*	*Unprepared*
Significant Benefit	**High Certainty**	**High or Moderate Feasibility**	**A1**	**A2**	**A3**
		Low Feasibility	**A4**		
	Moderate Certainty		**B**		
Zero to Small Benefit	High or Moderate Certainty		**C**		
Negative Benefit			**D**		
	Low Certainty		**L**		

Key Considerations for PHSI

- Ability To Screen
- Short-Term Follow-up
- Long-Term Follow-up
- Newborn Screening Program Organization
- *Data systems/Information Exchange*
- *Direct Costs*
- *Opportunity Costs*
- *Leadership and Motivation*

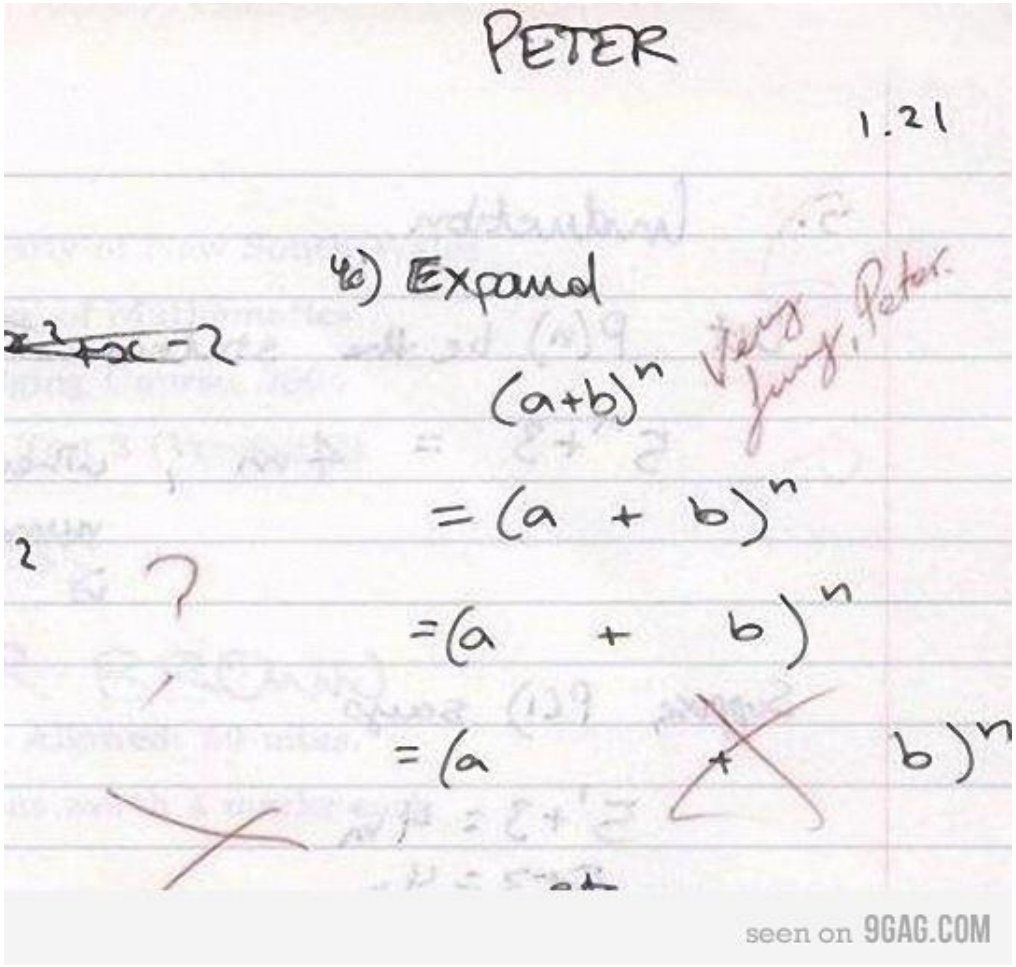
Key Stakeholders

- Newborn Screening Program Directors
- Newborn Screening Program Laboratory Directors
- Public Health Commissioners
- State Government Officials
- Laboratory and Clinical Specialists
- Primary care providers
- Payers

General Approach

- Focus on the features that would drive the Advisory Committee decision-making process to expand RUSP
- Consider General NBS and Condition-Specific issues separately
- Gather input from all states
  - *Gather additional information from states that have adopted or have attempted to adopt screening*
- Key point-of-contact from each state, who will work with others to respond to questions
- Use a standard approach to PHSI assessment for all conditions
  - *improves efficiency and consistency*
  - *allows comparisons*
  - *responsive to OMB requirements*

Peter's Rule to Expansion…



- ***Keep it Simple!***

Data Collection Approach and Sources

General NBS-related issues

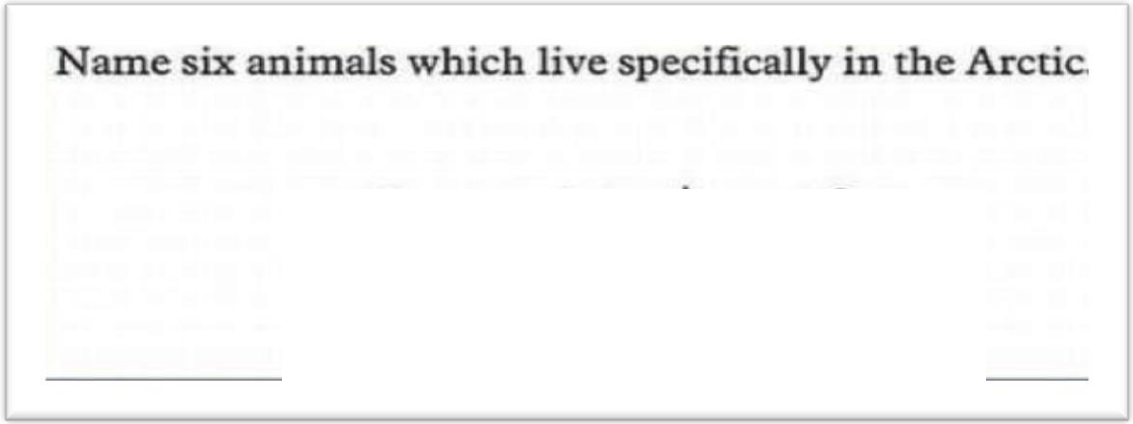
- ***Data Elements***
  - Process for adding conditions to state panels
  - Existing NBS infrastructure, laboratories, and workflow
  - Laboratory and reporting Systems
  - Short- and long-term follow up approaches and requirements
- ***Sources***
  - NewSTEPS Data Repository and Programmatic Surveys
  - Regional Collaboratives

Condition-specific NBS issues

- ***Data Elements***
  - Validated screening/ high throughput methods
  - Laboratory follow up, reporting, and diagnostic confirmation methods
  - Short- and long-term follow up needs
  - Treatment specialty centers and clinical guidelines
- ***Sources***
  - Evidence review findings
  - Web-based Surveys of states, Regional Collaboratives
  - In-depth Interviews with key program contacts from states with experience screening for the target condition

Condition Review Process Changes

- 3 Components
  - *Systematic Evidence Review*
  - *Population Benefit (i.e., Decision Analysis)*
  - *Public Health System Impact Assessment*
- 9-months to completion in the future
- More to cover in Less Time
  - ➢*Keep it Simple and Straightforward!*



Steps for the Condition-Specific PHSI Assessment

1. Work with the RCCs to
   - *Find out which states have/are/is planning to screen for the condition, so that we can expedite the interviews*
   - *Identify most appropriate survey respondent (DISCUSSION POINT)*
2. Identify the respondents from each state
3. Prepare educational material
   - *FAQ sheet*
   - *Webinar – to be recorded*
4. Field the survey
   - *Simple*
   - *Strong focus on what would help the AC make a decision*
   - *Reusable*
5. Conduct interviews with states that are implementing screening for the condition – concurrently with the fielded electronic surveys

Survey Discussion

Review of Public Health Impact Assessment Survey

PHSI Survey

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The purpose of this survey is to inform the Secretary of Health and Human Services Discretionary Advisory Committee about the ability to add newborn screening for particular conditions within your state.

Please refer to the [CONDITION] screening FAQ sheet and the information available at [LINK] to answer the following questions about the ability to add newborn screening for [CONDITION] within your state. Please also consult with others within your state to answer the questions you are unsure about.

1. Respondent Information
    a. Name
    b. Contact Information
    c. Job description
    d. State

2. Does your state currently include [CONDITION] as part of newborn screening, either as a part of the routine newborn screening panel or as any type of pilot evaluation?
    a. Yes=> Stop and schedule follow-up interview
    b. No

3. At any point within the last three years, has your state included [CONDITION] as part of newborn screening, either as part of the routine newborn screening panel or as any type of pilot evaluation?
    a. Yes=> Stop and schedule follow-up interview
    b. No

4. Has there been a state-level decision to add the target condition to newborn screening in your state?
    a. Yes=> Stop and schedule follow-up interview
    b. No

5. Which of the following conducts newborn screening laboratory analysis services for your state's newborn screening program?
    a. Your own state's public health or newborn screening laboratory
    b. A contracted regional newborn screening laboratory or other not-for profit laboratory.
    c. A contracted commercial laboratory

[LOGIC NOTE: 5a = NBS LAB, 5b or 5c = OUTSIDE LAB]

PHSI Survey

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6. Certain factors related to the [CONDITION] might make screening easier or more challenging in your state. Please let us know the degree to which these factors affect your ability to screen for condition in your state. In order to respond to these questions, assume that the condition has been authorized for addition to your state's panel and that funds are made available.

What is the impact of [Factor] on the adoption of screening for the [CONDITION] in your state?

Factor	Major Barrier	Minor Barrier	No Impact	Minor Facilitator	Major Facilitator	Unsure
Laboratory equipment needed to screen specimens for this condition[NBS LAB]						
Extent to which the laboratory protocol for screening for this condition has been demonstrated in other newborn screening programs [NBS LAB]						
Qualified technical staff within your laboratory to screen for the condition [NBS LAB]						
Availability of the screening test within the lab you contract with [OUTSIDE LAB]						
Priority of other ongoing newborn screening program activities (e.g., addition of other conditions, other quality improvements)						
Capacity of Laboratory Information Management System and its interface with the instrumentation						
Sufficient number of qualified newborn screening staff to notify and track newborn screening results						
Access to appropriate diagnostic services after a positive screen (e.g., diagnostic laboratory testing, clinical evaluations)						
Availability of specialists						
Availability of treatment for those diagnosed through newborn screening						
Cost of treatment for newborns diagnosed with newborn screening						
Expected clinical outcomes of patients identified by screening in your state						
Expected cost-benefit of screening in your state						
Advocacy for screening for this condition						
Other non-newborn screening public health priorities within your state						

PHSI Survey

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7. Are there other important barriers to screening for the [CONDITION]? [FREE TEXT RESPONSE]

8. Are there other important facilitators to screening for the [CONDITION]? [FREE TEXT RESPONSE]

9. What is the most significant barrier to newborn screening for the [CONDITION] in your state?[FREE TEXT RESPONSE]

10. What would most facilitate screening for the [CONDITION] in your state?[FREE TEXT RESPONSE]

PHSI Survey

Page 4

11. How long would it take to achieve the following assuming that [CONDITION] was added to your state newborn screening panel and funds were allocated today?

	Within 1 year	From 1 to 3 years	More than Three Years	Unsure
Select, develop, and validate the screening test within your laboratory[NBS LAB]				
Contract with a lab that has already validated screening[OUTSIDE LAB]				
Pilot test screening within your state				
Implement statewide screening for all newborns, including full reporting and follow-up of abnormal screens				

PHSI Survey

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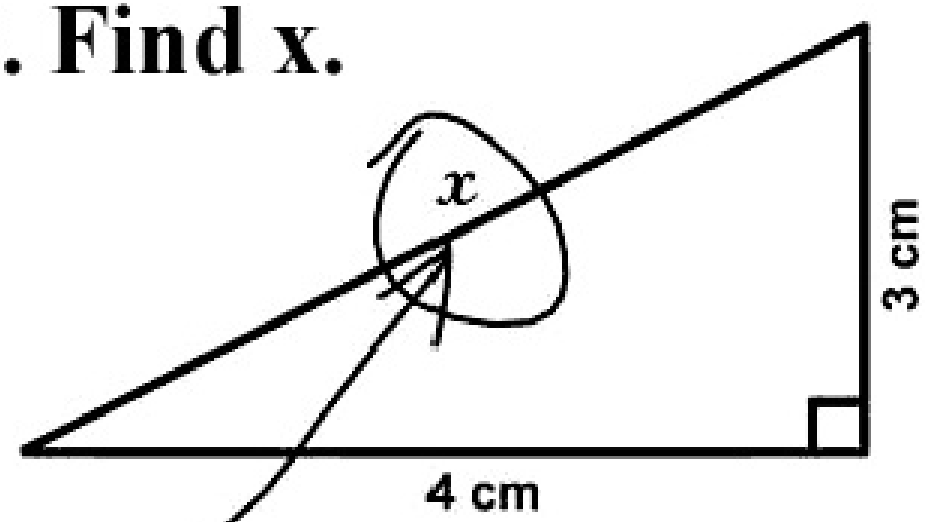
11. Is there any other information you would like to share with us regarding implementation of newborn screening for [Condition]? [Free Text Response]

12. Who did you consult with to answer these questions?
    a. State newborn screening laboratory experts
    b. Other newborn screening program staff
    c. State newborn screening advisory board
    d. Specialists in the condition
    e. Primary care providers
    f. Advocates within your state for screening for the condition
    g. Others: _____

Focus on Information Critical for the Committee's Decision Making

3. Find x.



Thank You!

Questions?

Presentation Contact:

Alex R. Kemper, MD, MPH, MS
alex.kemper@duke.edu