

RUSP Condition Nomination and Evidence Review Process

Approach and Timeline

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The Review Process: Four Focus Areas

1. Nomination
- 2. Systematic Evidence-based Review**
3. Decision Matrix
4. Review of Current Conditions on the RUSP

Recap of Topics Covered

- **April 2019:** Systematic evidence review
 - Case definitions
 - Outcomes measures
 - Treatment/intervention
 - Grading the evidence
 - Identifying and synthesizing unpublished evidence/data
- **August 2019:** Systematic evidence review
 - Cost assessment
 - Population-level modeling
 - Public health system assessment
 - Assessing values
- **September 2019:** Systematic evidence review
 - **Public Health System Impact Assessment**

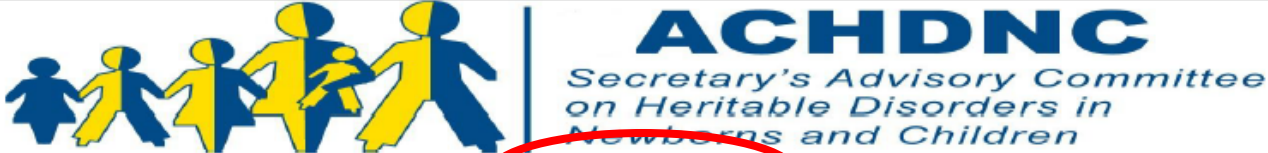


Public Health System Impact Assessment (PHSI)

- **Per the NBS Saves Lives Reauthorization Act of 2014, the Committee shall:**
 - “develop a model decision-matrix for newborn screening expansion, including an evaluation of the potential public health impact, including the cost of such expansion...”
- **The assessment of state newborn screening programs is intended to evaluate the entire integrated system needed for implementation of comprehensive newborn screening. Processes assessed include:**
 - authority
 - laboratory testing
 - interpretation
 - reporting
 - tracking
 - systems for assurance of diagnostic evaluation
 - evaluation of outcomes
- **The goal of the public health system impact assessment is to:**
 - Inform the Committee about the feasibility of screening, state readiness to implement new condition screening, and describe the costs of implementing a new condition screening.



Decision Matrix



NET BENEFIT/ CERTAINTY		READINESS			FEASIBILITY	
		Ready	Developmental	Unprepared	FEASIBILITY	
SIGNIFICANT Benefit	Certainty HIGH	A1 Screening for the condition has a high certainty of significant net benefits, screening has high or moderate feasibility. Most public health departments are ready to screen.	A2 Screening for the condition has a high certainty of significant net benefits and screening has high or moderate feasibility. Public health departments have only developmental readiness.	A3 Screening for the condition has a high certainty of significant net benefits and screening has high or moderate feasibility. Public health departments are unprepared for screening.	Feasibility	HIGH or MODERATE
		A4 There is high certainty that screening would have a significant benefit; however, most health departments have low feasibility of implementing population screening.				LOW
	Small to ZERO Benefit	MOD	B 1-4 There is moderate certainty that screening would have a significant benefit.			
NEG Benefit	Certainty MOD/HIGH	C 1-4 There is high or moderate certainty that adoption of screening for the targeted condition would have a small to zero net benefit.				--
		D 1-4 There is high or moderate certainty that adoption of screening for the targeted condition would have a negative net benefit.				--
--	LOW	L 1-4 There is low certainty regarding the potential net benefit from screening.				--



Feasibility

Key Features of Feasibility:

- availability of valid and reliable screening tests with adequate throughput to meet the needs of population-based deployment
- availability of systems to ensure quality implementation of the screening test (quality reagents and data-reporting systems)
- availability of quality-control and proficiency-testing samples
- adequate training programs for new technologies
- established approach for diagnostic confirmation available to newborn screening programs
- established approach to long-term follow-up, including treatment, available to newborn screening programs



Readiness

Key Features of Readiness:

- availability of resources for screening, diagnostic confirmation, and long-term follow-up, including financial resources
- availability of laboratory equipment, data systems, and expertise
- access to specialty care and treatments
- systems for data collection
- authorization for screening



PHSI Assessment: Current Approach

Public Health Impact - Population

- **Population modeling**
 - Quantitative approach to synthesis
 - Compares newborn screening to usual case detection
 - Dependent on inputs from the evidence review
 - Time horizon of the data
 - Availability of important outcomes

Public Health Impact - System

- **Cost assessment**
- **Surveying state newborn screening programs**



PHSI Assessment: Survey Tools Used to Assess the Impact on State NBS Programs

- Survey state newborn screening programs:
 - Online survey
 - NBS Programs are encourage to work with their partners to answer questions
- Follow-up interviews with NBS programs that have a mandate to screen, have begun (or have plans to begin) pilot testing screening for the condition, or have completed budget analysis for screening for the condition.

NBS Program Participation in Previous PHSI Assessments

	# programs invited to participate in PHSI Assessment	# programs responding	# invited to interview	# completed interviews	# invited to online PHSI survey	# completed online PHSI surveys	Non-respondents
Pompe	13	12 (92%)					1
MPSI	53	42 (79.2%)	3	3	50	39 (78%)	11 (20.8%)
XALD	53	37 (69.8%)	4	4	49	33 (67.3%)	16 (30.2%)
SMA	53	46 (86.8%)	5	5	48	41 (95.8%)	7 (13.2%)



Overarching Feedback on Survey Tools

- Surveys may not capture the difficulties of implementing a new condition
- The overall estimates of time it would take to implement a condition (e.g. 1 – 3 years) could be more informative
- Surveys may not account for possible impacts on primary-care physicians, specialists, and genetic counselors, etc.
- Public health programs may not know the answers for all of the questions
 - others that contribute into the newborn screening system may need to be engaged
- NBS programs may not know, at the time of the survey, what a long-term follow-up plan for a given condition would look like
- Survey questions are hypothetical and responses are subjective
- The surveys are approved and not modifiable for each condition



An Example of Feedback Informing Revisions to the Survey

- Feedback received regarding question 7 on the previous survey:
 - How accurate/valid are the answers?
 - Need more choices and/or just ask specific numbers?
 - What are things that may inhibit you from reaching that goal?
- Issues to address:
 1. Estimation of time needed for implementation activities.
 2. Capturing the barriers and challenges to implementing of screening.



ESTIMATION OF TIME TO IMPLEMENT A CONDITION

Revisions to the Survey – Timing of Activities

Previous Version:

7. How long would it take to achieve the following assuming that condition x was added to your state NBS panel and funds were allocated...?

- 1 year or less
- 1 to 3 years
- 3 or more years

Revised Version:

9. Please estimate the time it would take your NBS program to initiate screening for [condition x] in your state (i.e. get authority and funds to screen for condition x, go through administrative processes, meet with your state NBS committees and complete all activities needed to implement and commence screening for all newborns in your state).

- 12 months or less
- 13 to 24 months
- 25 to 36 months
- 37 to 48 months
- More than 48 months



Revisions to the Survey – Timing of Activities

10. The question above related to the overall timeline. We recognize some of the activities happen in tandem and some cannot begin until a previous activity has been completed. Please estimate the total time needed, in general, for each individual activity listed below within your NBS program. If needed, please consult with the laboratory and follow-up staff, medical professionals and specialists, prior to completing the survey.

Previous List of Activities

- ✓ Obtain and procure equipment for screening for [condition x]
- ✓ Hire necessary laboratory and follow-up staff
- ✓ Select, develop, and validate the screening test within your laboratory IF you ARE/are NOT multiplexing
- ✓ Add the screening test to the existing outside laboratory contract
- ✓ Pilot test the screening process within your state, after validation has taken place
- ✓ Implement statewide screening for all newborns, including full reporting and follow-up of abnormal screens after validation and pilot testing

Revised List of Activities

- ✓ Availability of funds to implement screening for condition x
- ✓ Meet with Advisory committees and other stakeholders
- ✓ Obtain and procure equipment for screening for [condition x]
- ✓ Hire necessary laboratory and follow-up staff
- ✓ Select, develop, and validate the screening test within your laboratory IF you are NOT multiplexing
- ✓ Select, develop, and validate the screening test within your laboratory IF you ARE multiplexing
- ✓ Develop a screening algorithm, follow-up protocols, and train follow up staff
- ✓ Set up reporting and results systems for added condition (e.g., LIMS)
- ✓ Collaborate with specialists and clinicians in the community to determine which diagnostic tests will be recommended upon identification of an out of range NBS result
- ✓ Add the screening test to the existing outside laboratory contract
- ✓ Conduct an internal validation study for [condition x]
- ✓ Pilot test the screening process within your state, after validation has taken place
- ✓ Implement statewide screening for all newborns, including full reporting and follow-up of abnormal screens after validation and pilot testing



CHALLENGES TO IMPLEMENTATION

Revisions to the Survey – Challenges to Implementation

Revised Question:

6. Please indicate the degree to which these factors impede or facilitate your ability to adopt screening for [condition x] in your state.

Previous Categories

- Will hinder implementation
- May hinder implementation
- No Impact
- May aid in implementation
- Will aid in implementation

New Categories

- **Major Barrier** – will prevent testing from being implemented effectively and/or timely
- **Minor Barrier** – may compromise testing so it is not performed effectively and/or timely
- **Minor Facilitator** – may allow testing to be done effectively and/or timely
- **Major Facilitator** – will allow testing to be done effectively and/or timely
- **Not Applicable**

Revised Question:

7. Please describe any additional factors that impede or facilitate adoption of screening for [condition x] in your state.

Discussion Questions for Today

1. Does the current PHSI Assessment approach (surveys and follow up interviews) capture all the information the Committee needs?
 - What additional information is needed?
2. Are there new or additional methods the evidence review process ought to include to gather information on the public health impact?
3. Which stakeholders are not represented in the current process?
 - How can all of the stakeholders contribute to the information?

