Duke Clinical Research Institute

Assessing Public Health System Impact: Summary of the Expert Advisory Panel Workgroup Meeting

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Expert Advisory Panel Workgroup on Public Health System Impact: Participating Stakeholder Groups

WORKGROUP STEERING COMMITTEE	STATE PUBLIC HEALTH DEPTS
Joseph A. *Bocchini*, Jr., *MD (Chair, AC/ LSUHSC at Shreveport)*	Sharmini V. Rogers, MBBS, MPH (MO DHSS)
Ned Calonge, MD, MPH (The Colorado Trust/U of CO Denver SOM)	Stephanie Mayfield Gibson, MD, FCAP (KY DPH)
Kay A. Johnson, MPH, Med (Johnson Consulting/Dartmouth SOM)	Janice Bach, MS, CGC (MI Dept Community Health)
Alex R. Kemper, MD, MPH, MS (Chair, CRW/Duke Univ Med)	**GENETIC COUNSELING**
PUBLIC HEALTH LABORATORY	Erica Wright, MS, CGC (U of CO-Denver, Clin Gen & Met)
Scott Becker (APHL)	**HERITABLE DISORDERS SPECIALISTS**
Jelili Ojodu, MPH (APHL)	Joseph Muenzer, MD, PhD (UNC-CH SOM)
Fred Lorey, PhD (CA DPH)	Kathryn Hassell, MD (U of CO Denver SOM)
Scott M. Shone, PhD (NJ DHHS, NBS Lab)	**PRIMARY CARE PROVIDERS**
Susan M. Tanksley, PhD (TX DSHS, Lab Operations)	Tracy Trotter, MD (San Ramon Valley PC Med Group)
NCC/REGIONAL COLLABORATIVE	Charlie Homer, MD, MPH (NICHQ/ AC)
Michael Watson, PhD (ACMG)	**PATIENT and FAMILY ADVOCATES**
Marci Sontag, PhD (NewSTEPS, APHL)	Don Bailey, PhD (RTI International, AC, Family Advocate)
Sylvia Mann, MS, CGC (HI Dept Health, Genetics Prog)	Jana Monaco (AC, Parent Advocate)
ETHICS	Cynthia Pellegrini (March of Dimes, Pub Pol & Gov Affairs)
Jeffrey P. Brosco, MD, PhD (Miami SOM/ So Region CMS, FL DOH)	
PUBLIC HEALTH/ IMPACT ASSESSMENT	
Mirelle Goetghebeaur, PhD (EVIDEM Collab, U of Montreal)	
Russell S. Kirby, PhD, MS (University of South Florida)	
EVIDENCE REVIEWS	
K.K. Lam, PhD (PL, CRW/Duke Univ Med)	
Linda A. Bradley, PhD, FACMG (Woment & Infants Hosp of RI/Brown U)	

Expert Advisory Panel Workgroup on Public Health System Impact: Federal Agency Representatives

FEDERAL AGENCIES
Michael C. Lu, MD, MS, MPH (MCHB/HRSA)
Joan A. Scott, MS, CGC (Gen Services Branch/MCHB/HRSA)
Debi Sarkar, MPH (Gen Services Branch/MCHB/HRSA)
Bonnie Strickland, PhD (DSCSPN/HRSA)
Cynthia F. Hinton, PhD, MS, MPH (CDC/NCBDDD)
Kellie B. Kelm, PhD (Ctr for Devices & Radiol Health/ US FDA)
Melissa Parisi, MD, PhD (IDDB/NICHD)
Carla Cuthbert, PhD, FCCMG, FACMG (NBSMBB/CDC)
Scott Grosse, PhD (NBSMBB/CDC)
Kara Contreary, PhD Candidate Ctr for CDC/Prev Effectiveness)

Meeting Approach

Participants – Total 38

- Represented key stakeholder groups involved in newborn screening.
- Select Advisory Committee and Condition Review Workgroup Members
- Key Federal Agency Representatives

Pre-meeting Preparatory Materials to Participants:

a) *condition review and decision-making process to expand RUSP,*
b) *major activities in newborn screening in the United States,*
c) *other models of review and decision-making for genetic testing and population-based health interventions through literature review.*

Meeting Procedures Followed by Facilitators:

- Brief review of preparatory material highlights
- Presentation of sample condition reviews to illustrate implementation issues
- Collaborative brainstorming and consensus methods to elicit 3 major aspects of PHSI:
  - *WHAT elements to consider*
  - *WHO should be asked*
  - *HOW information should be gathered (within time & resource limits)*

SACHDNC Decision Matrix

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**		

EAP PHSI Meeting Objectives

- Define Public Health System Impact (PHSI) Assessment
  - *WHAT, WHO, HOW*
- Identify how the PHSI Assessment can be used in decision making

WHAT Should the PHSI Assess? Key Considerations

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

Newborn Screening Program Organization

- Authorization process for adding a new condition
- Process for obtaining additional funds
- The role of public health in providing access (including coverage) after a positive screen for diagnostic services or treatment services for the child or family (e.g., referral for genetic counseling)
  - *Targeted condition*
  - *Incidental findings / Secondary conditions*
  - *Carriers*
  - *Genotypes of uncertain significance*
  - *Late-onset disease*
- Contextual factors
  - *Motivation for change and Leadership*

Ability to Screen: Many Considerations

- Screening methods
  - *Dried Blood Spots (DBS) vs. Point-of-Care (POC)*
- Laboratory considerations
  - *Validated screening method/platform that can be adopted*
  - *Time to analyze a specimen and time to report*
  - *Availability of quality-control materials and standards*
  - *New test vs. an extension of a process already in place*
  - *Equipment and supplies*
  - *Need for new laboratory employees*
- POC Considerations
  - *Validated screen*
  - *Ability to incorporate into flow*
  - *Need for training*
  - *Equipment and supplies*
  - *Need for new employees*

Ability To Screen: Laboratory-based NBS

- *Description of laboratory technology*
- *Evaluation of resources needed to implement (employees, including qualifications and expertise, materials, data system)*
- *Expected need for short-term follow-up*
- *Impact on laboratory screening process*
- *Impact on costs, including newborn screening fees*

Ability to Screen: Point-of-Care NBS Measures

- *Description of equipment needed for screening*
- *Evaluation of the effort requirement to provide the screening and report the results*
- *Evaluation of data system needs*
- *Description of the role of the NBS program, which will vary by state*
- *Expected need for short-term follow-up*
- *Evaluation of expected costs*

Short-Term Follow-up

- Follow-up
  - *Defined process / algorithm (vary by laboratory vs. POC NBS)*
  - *Public health personnel*
- Data infrastructure and Health Information Exchange
- Availability, accessibility, costs of diagnostic testing, including specialist services
  - *In state*
  - *Out-of-state*
  - *Existing / new collaborations*

Long-Term Follow-Up

- Data systems to monitor vs. service delivery
- Availability and accessibility of treatment
- Need for out-of-state services
- Need to follow those with
  - *Variants of unknown significance*
  - *Carriers*
  - *Presymptomatic or late-onset disease*
- Implications for NBS Program or Title V Programs

WHO Should be asked about PHSI? Key Stakeholders

- Newborn Screening Program Directors
- Newborn Screening Program Laboratory Directors
- State Department of Public Health Commissioners (to direct to key players)
- Laboratory and Clinical Specialists in pediatrics and condition
- Primary care providers

HOW should we gather information about PHSI?

- **General NBS**: Some data are not specific to the particular condition and are available from existing surveys (e.g., NewSTEPS)
  - *Process for adding a condition*
  - *NBS obligations for short- and long-term follow-up*
  - *Existing infrastructure/workflow*
- **Condition-specific NBS:** data will be obtained from published evidence, and used to both inform and survey the states

Using findings from the PHSI to guide DACHDNC recommendations

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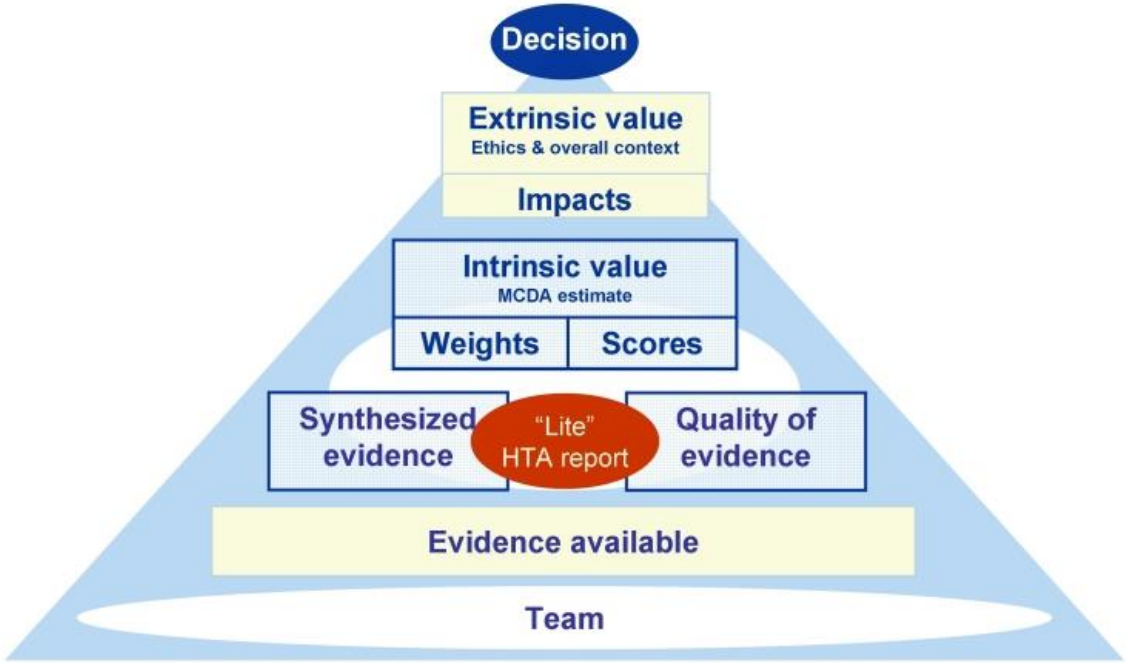
DACHDNC Use of the PHSI Assessment

- Assessment of feasibility and readiness
- Identification of gaps
- Roadmap for implementation for conditions added to the RUSP
- Recommendations for those conditions that lack feasibility or readiness

Multi-criteria / Multi-perspective Decision Analysis

- EVIDEM (www.evidem.org)



Proposed Process for PHSI Assessment

1. The CRW identifies a list of PHSI questions
   - *General*
   - *Condition specific*
2. The DACHDNC makes recommendations about these PHSI questions for each condition.
3. The CRW develops a final list of PHSI questions and surveys the DACHDNC about the weighting of the questions.
4. The CRW prepares a report outlining the final PHSI questions and the relative weights.
5. The CRW prepares the PHSI report based on all PHSI questions, with focus on those with higher weight.
6. The DACHDNC makes decisions regarding feasibility and readiness, guided by the agreed upon PHSI questions and weighting.

This process would have to begin after the initial evidence review presentation to the DAHDNC

Next Steps

- Pilot the development of PHSI questions and weighting with the EAP.
- Finalize the summary report of the EAP based on the meeting and pilot work, which will be used as part of the methods used by the CRW.
- Use the revised approach with MPS-1.