

# Follow-up and Treatment Subcommittee Report

April 19, 2013

Carol Greene, MD, Chair

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# Update on “EHDI” Project

- Lessons learned from EHDI applicable to other point of care NBS
- Presentation on agenda this meeting

# Update: Outcomes After NBS Project

- Revised focus and new title “A Framework for Assessing Outcomes from Newborn Screening”
  - Harmonization of key questions/data needs
  - Consideration of data sources and gaps
  - Sickle Cell as an example to ensure framework is useful
- Work in progress, draft framework

FRAMEWORK ELEMENT (All Conditions)	CONDITION SPECIFIC RESPONSES	CONDITION SPECIFIC POTENTIAL MEASURE	POTENTIAL DATA SOURCES*	KEY COMPONENT ADDRESSED**
<b>DETECTION AND DIRECT OUTCOME(S) OF INTERVENTION</b>				
Condition Detected by NBS				
Time for disease Confirmation				
Justification for NBS (early childhood outcomes)				
<b>ACCESS TO CARE</b>				
Primary care provider				
Specialty care provider				
Family satisfaction with care				
<b>OTHER KEY OUTCOMES</b>				
Prevention of major childhood disease complications				
Assessment of adequate growth				
Disease-modifying therapies				
Educational level for age				
Genetic services				

\*See Hinton et al 2011

\*\*LOCAL, STATE, REGIONAL AND/OR NATIONAL DATA SOURCES

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<b>DETECTION AND DIRECT OUTCOME(S) OF INTERVENTION</b>				
Condition Detected by NBS				
Time for disease Confirmation				
Justification for NBS (early childhood outcomes)	Use of prophylactic penicillin	<ul style="list-style-type: none"> <li>▪ % infants prescribed penicillin by 4 months of age**</li> <li>▪ % children at age 5 on penicillin</li> </ul>	NBS programs, RuSH ***HRSA- supported surveys	
<b>ACCESS TO CARE</b>				
Primary care provider				
Specialty care provider				
Family satisfaction with care				
<b>OTHER KEY OUTCOMES</b>				
Prevention of major childhood disease complications				
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\*\*\*RUSH data has significant limits and gaps

# Outcomes Framework Next Steps

- Compare draft framework with other existing standard data element sets
- Revise framework; get input from colleagues/ other stakeholders and revise framework
- Explore gaps in data sources, including attention to the issue of the denominator
- Bring revised framework and draft paper to full committee next meeting

# Follow-up and Treatment Subcommittee: Forecast

- The SACHDNC will consider challenges in point-of-care newborn screening by examining lessons learned from Early Hearing Detection and Intervention (EHDI) that may be applicable to Critical Congenital Heart Disease (CCHD) Screening and other point of care screening. Issues considered will include integration of POC screening with the blood spot screening program; and the roles of public health in clinical POC testing, reporting and follow up. The Committee will develop a framework that can be used to assess the outcomes of newborn screening. To be certain that any framework will be practically useful, experts in Sickle Cell Disease will be involved to apply the framework to SCD as an example. The framework will be applicable to other conditions with respect to types of data elements and data sources needed to know if the goals of screening are achieved. The Committee will also begin to explore the impact of the Affordable Care Act on individuals and families with heritable conditions.