

Follow-up and Treatment Subcommittee Report

February 1, 2013

Carol Greene, MD, Chair

Chris Kus, MD, MPH, Co-Chair

Reports

- Information to Subcommittee on relevant ongoing projects:
 - Information from S. Berry about HRSA Priority 2-NIH IBEM-IS Project
 - Information from A. Brower about NBSTRN, with focus on the Longitudinal Pediatric Data Resource (LPDR)

Update on “EDHI” Project

- Newborn Hearing Screening Survey – Brad Therrell
- Lessons learned from EDHI applicable to the implementation of CCHD/point-of-care NBS, and other future NBS activities

Newborn Hearing Screening Program Survey (Brad Therrell)

- Survey completed – 4 questions
- Shows states that obtain hearing screening results on the newborn bloodspot screening card & those that obtain results through the electronic birth registration process
- Subcommittee members have received the survey results for further analysis

Lessons learned from EHDI Project applicable to other NBS (point-of care)

- Some general themes have been outlined
 - Other Screening should strive to link with other State screening efforts (bloodspot, EHDI)
 - State Health Department should play a leadership role in integrating screening data systems
 - Appropriate financial support (federal & state) will be needed to develop the screening system (add CCHD)
- Small working group will develop another draft of “Lessons Learned” – this will be completed by March 15, 2013

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**
DETECTION AND DIRECT OUTCOME(S) OF INTERVENTION				
Condition Detected by NBS				
Time for disease Confirmation				
Justification for NBS (early childhood outcomes)				
ACCESS TO CARE				
Primary care provider				
Specialty care provider				
Family satisfaction with care				
OTHER KEY OUTCOMES				
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

FRAMEWORK ELEMENT (All Conditions)	CONDITION SPECIFIC RESPONSES	CONDITION SPECIFIC POTENTIAL MEASURE	POTENTIAL DATA SOURCES*	KEY COMPONENT ADDRESSED**
DETECTION AND DIRECT OUTCOME(S) OF INTERVENTION				
Condition Detected by NBS				
Time for disease Confirmation				
Justification for NBS (early childhood outcomes)	Use of prophylactic penicillin	<ul style="list-style-type: none"> ▪ % infants prescribed penicillin by 4 months of age** ▪ % children at age 5 on penicillin 	NBS programs, RuSH ***HRSA- supported surveys	
ACCESS TO CARE				
Primary care provider				
Specialty care provider				
Family satisfaction with care				
OTHER KEY OUTCOMES				
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

***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

Update Medical Foods

- Update from Christine Brown:
 - No federal authority to require state coverage
 - No right of appeal if not covered by state
 - Advocacy community plans to work with Congress to reintroduce Medical Foods Equity Act
- Update from Cathy Camp:
 - NIH projects focus on research and on access
 - Technical support for professional organizations working on evidence-based guidelines
- Medical Foods Paper in press

Future –FU&TR Subcommittee

- Timeline for EDHI/POC lessons-learned project
- Timeline for the Framework for Outcomes of NBS – data elements and data sources project
- Ongoing interest in medical foods (no active sub-committee project)
- Looking forward to the presentations planned for the next SACHDNC meeting on impact of ACA and essential health benefits decisions on population of children with heritable conditions