

DACHDNC

Follow-Up and Treatment Subcommittee

May 30, 2014

Charles Homer, MD, MPH, Chair

Christopher A. Kus, MD, MPH, Co-Chair

Carol Greene, MD Outgoing Chair/Reporter

Report

- Review of Charge and Priorities
- Review of work since last meeting
- Discussion of document submitted for Committee review
- Presentation to Committee of possible future projects

Follow-Up and Treatment Subcommittee Roster

OFFICIAL MEMBERS

- Deborah Golant Badawi, MD +
- Susan A. Berry, MD
- Robert Bowman, MS
- Christine S. Brown, MS
- Denise Dougherty, PhD *
- Carol Greene, MD +
- Kathryn Hassell, MD
- Charles, Homer, MD **(Chair) ***
- Celia I. Kaye, MD, PhD
- Alex R. Kemper, MD, MPH, MS
- Christopher A. Kus, MD, MPH **Co-Chair +**
- Sylvia Mann, MS, CGC
- Jana Monaco
- Robert J. Ostrander, MD
- Brad Therrell, PhD
- Alexis Thompson, MD, MPH *
- Andrea Williams *

* **Committee Member**

+ **Organizational Representative**

OTHER EXPERTS

- Amy Brower, PhD
- Kathryn Camp, MS, RD, CSP
- John Eichwald, MA, FAAA
- Lisa Feuchtbaum, DPH, MPH
- Debra Freedenberg, MD, PhD
- Terese Finitzo, PhD
- Nancy C. Green, MD
- Kathy B. Harris, MBA
- Cindy F. Hinton, PhD, MS, MPH
- Rani Singh, PhD, RD
- Marci Sontag, PhD
- Alan E. Zuckerman, MD, FAAP

HRSA MCHB DSCSHN

- Irene Forsman, MS, RN **(ISB)**
- Edward (Donnell) Ivy, MD **(GSB)**
- Marie Mann, MD **(ISB)**
- Jill Shuger, ScM **(GSB)**

(GSB/Genetic Services Branch)

(ISB/Integrated Services Branch)

Subcommittee Charge (as it was revised September 2011)

Engage in a multi-step process that:

- Identifies barriers to post screening implementation and short- and long-term follow-up, including treatment, relevant to newborn screening results;
- Develops recommendations for overcoming identified barriers in order to improve implementation and short- and long-term follow-up, including treatment, relevant to newborn screening results; and
- Offers guidance on responsibility for post-screening implementation and short- and long-term follow-up, including treatment, relevant to newborn screening results.

Subcommittee priorities determined by Committee

- Priority A: “Screening program implementation”
- Priority B: “Closing gaps in systems of care”
- Priority C: “Real world impacts and outcomes”

PRIORITY A: Screening program implementation

- Project assigned: Assessing challenges of new Point of Care tests.
- Project recently completed: *Some Lessons Learned from Early Hearing Detection and Intervention (EHDI) that may be applicable to Critical Congenital Heart Disease (CCHD) Screening*

PRIORITY B: Closing Gaps in Systems of Care

- No specific project (yet) assigned; however Committee specifically asked that roles and responsibilities in LTFU be considered in the following ways:
 - As part of case studies, include focus on learning what are the current (and variable) roles and responsibilities in LTFU
 - Make sure that all LTFU sub-committee projects look at roles and responsibilities.

PRIORITY C: Real world impacts and outcomes

- Project assigned to:
 - Explore the extent to which we can document improved clinical outcomes to determine whether we are realizing the potential of NBS.
 - Includes evaluation of the impacts of variability in clinical care, in notification of and action regarding carrier status, in use of EHRs, gaps in services for S Cell Dx patients, etc.

Subcommittee Work Since Meeting January 2014

- Monthly phone conference calls working on the projects
- Additional phone calls and active e-mails of “writing group” for the Priority C project

PRIORITY A

Screening Program Implementation

Project for Priority A – Assessing challenges of new Point of Care tests. COMPLETED case study:

Some Lessons Learned from Early Hearing Detection and Intervention (EHDI) that may be applicable to Critical Congenital Heart Disease (CCHD) Screening

PRIORITY C

A Framework for Assessing Outcomes from Newborn Screening: Do we know if we are achieving the promise of NBS?

- Not to duplicate efforts occurring at HHS (*or anywhere else*). Focus is on developing key questions and understanding data sources, and to identify gaps. Process:
 1. Create a framework
 2. Use S Cell as example to be test framework and revise as needed until framework includes essential data types and permits mapping of data sources and gaps
 3. Test (and revise) framework against other conditions so that final framework can be applied to future understanding the real world impact of NBS for any condition.

PRIORITY C

A Framework for Assessing Outcomes from Newborn Screening: Do we know if we are achieving the promise of NBS?

- Draft provided to Committee for review and comments with text complete and some edits still required on tables
 - Framework and basic manuscript as previously reviewed by Committee
 - Some new materials (PKU table and driver diagram) to lay groundwork for practical use of the framework
 - From May 29 Subcommittee meeting
 - 4 edits to text
 - One minor edit to the “Driver Diagram”
- Discussion

Edits from May 29 meeting #1:

Bottom of page 3/end of introduction add:

- *These very different conditions bring different perspectives to developing and testing the framework with respect to outcomes. For both conditions there are also different types of and locations of data to test how the framework can be used to assess data availability.*

Edits from May 29 meeting #2:

Middle of page 4 insert:

-with a driver diagram model (3). *This identifies the primary drivers, which are system components or factors that contribute directly to the outcomes.* The purpose of introducing....
- And several lines later delete “Secondary drivers that are needed to achieve the primary drivers were also considered.”

Edits from May 29 meeting #3:

Page 5 first paragraph:

- Add/edit “*Consistent with the prior work of Kemper et al and Hinton et al, the four primary drivers we propose are: 1) rapid and reliable.....*” (*inserting numbers to clarify the four drivers*)
- And remove the next sentence with information about secondary drivers

Edits from May 29 meeting #4:

- Page 25 in Summary add:
- “... to populating those measures. *The framework will be useful for any condition, allowing for customization of condition-specific and program-specific outcomes. It is a tool for evaluation of whether necessary data exists, or whether there are gaps pointing to need for additional data collection. Having the framework built with a driver diagram also provides a vision for....*

Potential Projects in/ Priority Areas for Committee Activity Discussion

- Build off of “Framework” Manuscript
- Describe the public health/clinical interface
- Build program improvement capacity

Recommended Future Committee Activity

- ❖ Describe the current public health/clinical care interface

Consider profiling several states as to their public health/clinical care interface – how do things work in the profiled states?