

# **DACHDNC**

## **Follow-Up and Treatment Subcommittee Report** **September 11, 2014**

**Chairperson, Charles F. Homer, MD, MPH**  
**Committee Member**

# Follow-Up and Treatment Subcommittee Roster

## OFFICIAL MEMBERS

- Deborah Golant Badawi, MD +
- Susan A. Berry, MD
- Christine S. Brown, MS
- Denise Dougherty, PhD \*
- Carol Greene, MD +
- Kathryn Hassell, MD
- Charles, Homer, MD \* Chairperson
- 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
- 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)

## Follow-up and Treatment Subcommittee Charge (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.

## PRIORITY C: Real World Impacts and Outcomes

- 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.

## PRIORITY C: Real World Impacts and Outcomes

- *A Framework for Assessing Outcomes from Newborn Screening: Do we know if we are achieving the promise of NBS?*
  - *Defined key outcomes: survival and well being (WHO definition of Health) including disparities*
  - *Defined 4 “drivers”:*
    - *Rapid and Reliable Diagnosis*
    - *Evidence based therapeutic and habilitative care*
    - *Coordination and integration for services*
    - *Continues improvement and knowledge generation.*
  - *Illustrated with measures reflecting these drivers and outcomes for SCD and PKU*

## Priority C: Real World Impacts and Outcomes

- New Activity Requested/Approved by Committee based on NBS Outcomes Manuscript
- Operationalizing the Framework: Models of systems with capacity to monitor outcomes and improve care.
  - Public health system
  - Clinical delivery systems
- Describe the public health/clinical interface
  - Consider profiling several states as to their public health/clinical care interface – how do things work in the profiled states
- Build program improvement capacity to improve care based on data

# Actions for Subcommittee

- Coordinate more effectively with Regional Genetics Collaboratives
- Through RGC's and the NCC:
  - Clarify which states have LTFU systems in place (to monitor and improve LTFU)
  - Identify barriers to more widespread implementation of such systems
  - Identify states that have promising systems:
    - Preliminary: MA, CA, MI, IN, ?NY, ?RI

# For states with promising systems

- Determine what capacity these states have to implement framework
- Understand how states were able to achieve what they have achieved
- Consider how such capacity might be extended elsewhere
- Additional considerations:
  - That in focusing on what IS we don't lose track of what can be...and is now under development.
  - That we connect to and accelerate adoption of LPDR – longitudinal pediatric data resource



# Ongoing Concerns

- That our efforts NOT focus on data and measurement to exclusion of USE of the data to monitor and improve care and outcomes.
- That we not lose focus on broader charge of subcommittee, i.e., identify and address barriers to long term treatment, including supply of and access to appropriate care and expertise.

# Workgroup includes

- Sylvia Mann
- Lisa Feuchtbaum
- Susan Berry
- Celia Kaye
- Kathy Hassell
- Ann Comeau
- Anne Chow
- Deborah Golant Badawi
- Susan A. Berry
- Amy Brower
- Rani Singh
- Terese Finitzo