SACHDNC Follow-Up and Treatment Subcommittee

Report to the SACHDNC

September 14, 2012

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Subcommittee Membership

- Membership Roster & Round Robin
- Changes in subcommittee membership:
  - Thank you and farewell to Michelle Fox, MS, CGC*
  - Welcome to Deborah Badawi, MD
  - Welcome to Kathryn Hassell, MD
- Appreciation for volunteers assisting with the various projects

* Once a member – always a volunteer!

Updates – some direct result of subcommittee efforts

- Brad Therrell, PhD reported on publication of the work on connecting newborn screening blood spots and birth certificates
- Susan Berry, MD reported on status of revising for publication manuscript on coverage of medical foods and supplements
- Rani Singh, RD, PhD reported on NBS-connect gone live
- Susan Berry, MD reported on activity of the long-term follow up data and outcomes project
- Nancy Green, MD reported on publication of paper on key considerations for point of care screening of newborns

Subcommittee work since last meeting:

- Regular phone conference calls
- Focus on priority areas and projects previously vetted by full committee
- Project development - subcommittee members and volunteers formed workgroups
  - EDHI/point of care screening implementation (“Priority A” project)
  - SS/S trait (“Priority C” project)

PRIORITY B: Closing gaps in systems of care

- “Project” – Roles and Responsibilities in LTFU:
- As part of case studies, include focus on learning what are the current (and variable) roles and responsibilities in LTFU for children with hearing impairment or sickle cell (disease or carrier). Making sure that all our projects look at Rs & Rs.

PRIORITY A: Screening program implementation

- Project 1 – Assessing challenges of new Point of Care tests. Begin with hearing screening follow-up as a case study.

- Project 2 – Ongoing evaluation of CCHD implementation - will work with HRSA-funded Regional Collaboratives

Point of care implementation project

- Early stages but moving quickly, will be focus of next call, report at next Adv Comm meeting
- Therrell and White reported on review of current status of reporting/communication after hearing screening
- Zuckerman discussed EHR issues around POC screening
- General discussion issues and project goals
  - What lessons does EDHI experience offer for other (different) POC screening
  - Boyle suggests begin with POC key issues paper and develop implementation road map

PRIORITY C: Real world impacts and outcomes #1

- Project – Sickle Cell case study –
- 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 #2

- Not to duplicate efforts occurring at HHS (*or anywhere else*). Focus is on developing key questions and understanding data sources, and to identify gaps. Goal is both to understand the S Cell case and to make sure the case study of S Cell is leveraged as an opportunity to develop approaches that can be applied to future studies for understanding the real world impact of NBS for other conditions.

“Sickle Cell” Project

- Hinton and Green presented matrices developed to help organize and review
  - Questions
  - Data sources
- General discussion of issues, goals, concerns that public/families may have about privacy, use of *and study of use of* EHR, &need for
  - HARMONIZATION and
  - AVOIDANCE OF DUPLICATION

“Sickle Cell” Project

- Discussion of issues around selecting outcomes & indicators, and data availability
    - Different issues in Sickle Cell and S trait
    - Limits on data availability
        - Data on individuals vs aggregate
        - Privacy/public health & transparency considerations
    - Include S Thal, not other Hemoglobinopathies
    - Include focus on/issues relevant to patients/families and psychosocial outcomes

EXAMPLE/Option

Modified from late-night meeting of WG members - WORK IN PROGRESS!

Ongoing work on Approach

- Select for tracking key indicators including process and outcomes
  - Evidence-based/widely agreed upon by experts (harmonization)
  - Practicable to perform in clinical care and to track by EHR

Key treatment indicators for SCD

1) Penicillin prophylaxis initiated early (when?)
2) Pneumococcal vaccination completed by age 3
3) TCD (trans-cranial doppler) screening by age 2 and annually

4) Consideration for hydoxyurea]

Sentinel adverse events for SCD

1) Stroke
2) Sepsis
3) Acute chest syndrome
4) Death

Subcommittee Plans for Next Steps

- Discussion of possible final products and outcomes of S Cell and S trait project –
  - Useful in future decisions about implementing NBS,
  - Useful in designing future data collection in LTFU,
  - Useful to promote development of SIMPLE project that would give a focused look at effectiveness of sickle cell newborn screening in improving health and contribute to Quality Improvement efforts
  - White paper? RFA(s)? Other

Immediate Plans for Next Steps

- Identify other key national groups and ongoing efforts and understand the available data sources
- Identify gaps in information currently tracked (what key questions cannot be answered with existing data sources and strategies)
- Explore harmonization of key questions, health and other outcome indicators, data elements, strategies
- Workgroup meeting planned, report at next Adv Comm meeting