

Follow up and Treatment Subcommittee January 26, 2012 Report

Coleen Boyle, PhD, MS



Medical Food Activities

□ NIH Activities in IEMs (K. Camp)

- Nutrition and Dietary Supplement Interventions for IEM, Stakeholders Workshop
 - Gaps in safety and efficacy of nutritional treatments
- PKU Scientific Review Conference, 2/23-24/2012
 - Update to NIH consensus statement of 2000

□ Essential Benefit Package – medical foods (C. Brown, NPKUA)

- HHS held regional listening sessions – medical foods discussed
- Pre-bulletin – flexibility to states but they must choose among 4 options
 - Decision on coverage rests with states
 - Next steps – monitor implementation at state level

□ Evaluation of insurance coverage, RC survey (S Berry)

- Descriptive study of use foods and limits of ins coverage

CCHD Implementation -- MD (D. Badawi)

□ Convened an expert panel:

- Hospitals should follow the protocol of the ACHDNC
- Birth hospital is charged with screening and f-up and + screens
- All hospitals have capacity for screening and must establish the capacity for f-up (e.g. telemedicine or transport)
- Hospitals responsible for protocols for f-up and clinical management
- Health Dept responsible for surveillance data on screening
- Education should be provided to consumers, clinical staff and community providers
- Main costs to hospitals – staff time to screen and track results (broad sense)
- Main cost to state– infrastructure for evaluation (surveillance)

Committee Priorities

- ❑ **Overarching theme:** *Disconnect---mandate for NBS yet a lack of a mandate for f-up and treatment*
- ❑ **What to do about this?**
 - Monitor implementation
 - Clarify roles and responsibilities in f-up and treatment
 - Illustrate with specific examples: SCD, CCHD and SCID
 - Federal gov't investment--- alignment with the gaps
- ❑ **Identifying the 'cost of providing care' – critical information for decision makers**
- ❑ **Methods/process need to prioritize the work of the subcommittee**

Update on Federal Activities Newborn Screening for CCHD



NIH (NHLBI and NICHD)

- ❑ NHLBI provided access to the common nomenclature tool for congenital cardiovascular malformations, the **International Pediatric and Congenital Cardiac Code (IPCCC)** used in NHLBI-funded pediatric cardiovascular studies.
- ❑ NICHD looking into tools for assessing psychosocial burden on families resulting from screening.
- ❑ NHLBI has assessed the current practices related to CCHD screening in the 9 clinical sites of the **Pediatric Heart Network**. The PHN is a potential venue for conducting evaluations of aspects of screening, in conjunction with other federal partners.



HRSA

- ❑ **Funding the demonstration projects for implementation of CCHD in the states (FOA recently announced)**
 - 6 projects will be funded for 3 years
- ❑ **Assisting the NBS Clearinghouse to work with NHLBI and other stakeholders to have the most accurate educational materials on the website.**
- ❑ **Working with National Newborn Screening Genetics Resource Center to provide State technical assistance as requested**

CDC

❑ Evaluate state surveillance and tracking

- National Birth Defects Prevention Network surveyed all 50 states
- EpiAid in NJ– analyze data flow and tracking in hospital; f-up on failed screens
- AMCHP webinar – March/April;

❑ Conduct a cost-effectiveness analysis of newborn screening

- NJ: estimate time spent by birthing center staff in screening
- develop a cost-effectiveness model of newborn screening for CCHD

❑ Leverage electronic health records

- CDC created an EHR workgroup for BD; working with external partners