

# ***Subcommittee on Treatment and Follow up***

## **Advisory Committee on Heritable Disorders in Newborns and Children**



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# Subcommittee Members

- Alan Hinman
- Jill Levy Fisch
- *Celia Kaye*
- *Susan Berry*
- *James Figge*
- *Carl Cooley*
- *Fred Lorey*
- Coleen Boyle
- Denise Dougherty
- Christopher Kus
- Mike Watson
- Alex Kemper  
(consultant)
- Brad Therrell  
(consultant)
- Jill Shuger



# Update of Activities

- Long Term Follow up (LTFU):
  - Roles and Responsibilities Position paper under development by Kemper et al.
- Medical Foods:
  - June 2008 meeting of experts
    - ◆ Committee letter outlining possible legislative actions
  - Survey in 3 states – full implementation 3/09



# Standardizing Data Elements for LTFU

- **Objectives of the Meeting:** To begin discussion on how to develop a common set of variables that can address the information needs of assure optimal NBS long term follow up
  
- Information needs for the four components of LTFU (Kemper et al. 2008)
  - Care assurance and coordination
  - Quality improvement
  - Evidence-based treatment/management
  - Research
  
- Information needs of the major participants in LTFU
  - Healthcare
  - Consumers
  - Public health



# Meeting Summary

- Presentations:
  - Summary of LTFU data activities (blood spot and EHDI) by HRSA, CDC and NIH grantees
  - Health Information Technology Infrastructure
- Discussion:
  - Harmonize case definitions
  - What are the questions?
  - Standardize data items
    - ◆ Level of resolution
  - More details on ongoing activities



# Discussion and next steps

- Define what are the critical questions
- Define minimum data set
- Identify areas where the lack of standards poses a problem (e.g., case definition)
- “Use Case” for long-term follow up
- *What is the role of the subcommittee work vs. work of the Agencies*