

**Subcommittee on Treatment  
and Follow up**

**Advisory Committee on Heritable Disorders  
and Genetic Disease in Newborns and  
Children**



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# Subcommittee members

- Coleen Boyle
- Christopher Kus
- Denise Dougherty
- Carol Greene
- Javier Aceves
- Brad Therrell
- Jill Fisch
- James Newton
- Alan Hinman
- Joseph Telfair
- Julie Miller
- Jill Shuger



# Activities on Long Term Follow up (LTFU)

- Develop a position paper
  - Goal, definitions and major components
- Stakeholders input into the development of the position paper (4/18/07)
  - Major perspectives/systems impacted – individual/family, primary care, specialty care, public health, financial and regulatory, health information systems
- White paper developed by Kemper et al.
  - Endorsement by ACHDGDNC
  - Publication in Genetics and Medicine



# Components of White Paper on LTFU

- Goal – to achieve the best possible outcome for children and their families
- Definition – chronic disease management, condition-specific treatment, and preventive care
- Components
  - Evidence-based treatment
  - Coordination of care
  - Continuous quality improvement
  - New knowledge discovery



# Next Steps -- LTFU

- Implementation phase
  - Roles and Responsibilities:
    - ◆ Family
    - ◆ Health sector – primary and specialty care
    - ◆ Public health sector
  - Develop into a position paper
- Identifying various effective models – beginning with perspective of public health ??



# Medical Foods and Formulas

- Purpose: to reduce financial barriers to families in obtaining medical foods/formulas
- Activities:
  - ***Survey of families to get better information on need***
  - Explore state legislation and its impact
  - Evaluate existing professional policies on medical food requirements/need for formula/food coverage



# Medical Foods and Formula—Survey Tool

- Expert Panel (survey development)
- Focus groups of parents (survey development)
- Implementation in two regions (survey validation)





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