Evidence-Based Reviews for Newborn Screening

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The Nomination Process

- Step #1: The Nomination Form
- Step #2: Federal administrative review
- Step #3: Review by ACHDGDNC
  A) ACHDGDNC review
  B) External evidence-based review
  C) ACHDGDNC review and decision
Nominations and Review

- Nomination Form
- Federal Administrative Review
- Advisory Committee
- Evidence Review Group
- Recommendations to the HHS Secretary
Nomination Form

- Reviewed at October 2006 meeting
  - Affirmed form and process
    - Does the information clearly define a disease
    - What is the prevalence of the disease (in different populations)
    - Can the condition be identified reasonably well in screening
    - Are there actions after screening that can lead to positive outcomes
Refining the Nomination Form

- Definition of several terms
  - Accuracy (test)
  - Available (test)
  - Efficacy (treatment)
  - Urgency (treatment)
- Sensitivity and specificity floors
- Evidence regarding costs
- Harms of screening
Pilot studies

- Review of nomination form and information by ACHDGDNC may indicate need for more population data prior to evidence review, e.g.,
  - Testing and treating a condition in one State using another State as a control
  - Better evidence of prevalence
  - Screening effectiveness in population application
Issues in Evidence Review

- Rare conditions
  - Lack of randomized trials in many cases
  - Limited information on costs and benefits across all potential outcomes (ie, true and false positives and negatives)

- Access to evidence
  - FDA trials
  - Proprietary data
Evidence Group

- Core evidence group staff
  - Project director (epidemiology/methods)
  - Public health
  - Consumer
  - Cost/benefit analysis
  - (General) genetics

- Assisted by members of ACHDGDNC and *ad hoc* expertise for specific disorder(s)
  - Clear conflict of interest policy

- External advisory group – broader national representation – additional expertise in review methods, genetics, and health care providers
Evidence Timeframe

- Initial work to frame any remaining questions in nomination form, confirm definitions with ACHDGDNC members
- Fall AC meeting to prioritize conditions for in-depth systematic reviews
  - Current nominations reviewed by MCHB staff
  - Additional solicitation from community
- Evidence group to carry out reviews for spring meeting
Evidence Reviews

- Review evidence regarding
  - Condition (prevalence, natural history, different forms of condition)
  - Screening and diagnostic testing
  - Treatment (risks, benefits; applicability to what condition groups)
- Decision analytic framework, addressing harms and benefits
- Indicate where evidence is absent and what information would be most critical
- Presentation of evidence in summary and table form for AC review
- All decisions by AC – evidence group will not make recommendations