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# Evidence-Based Reviews for Newborn Screening

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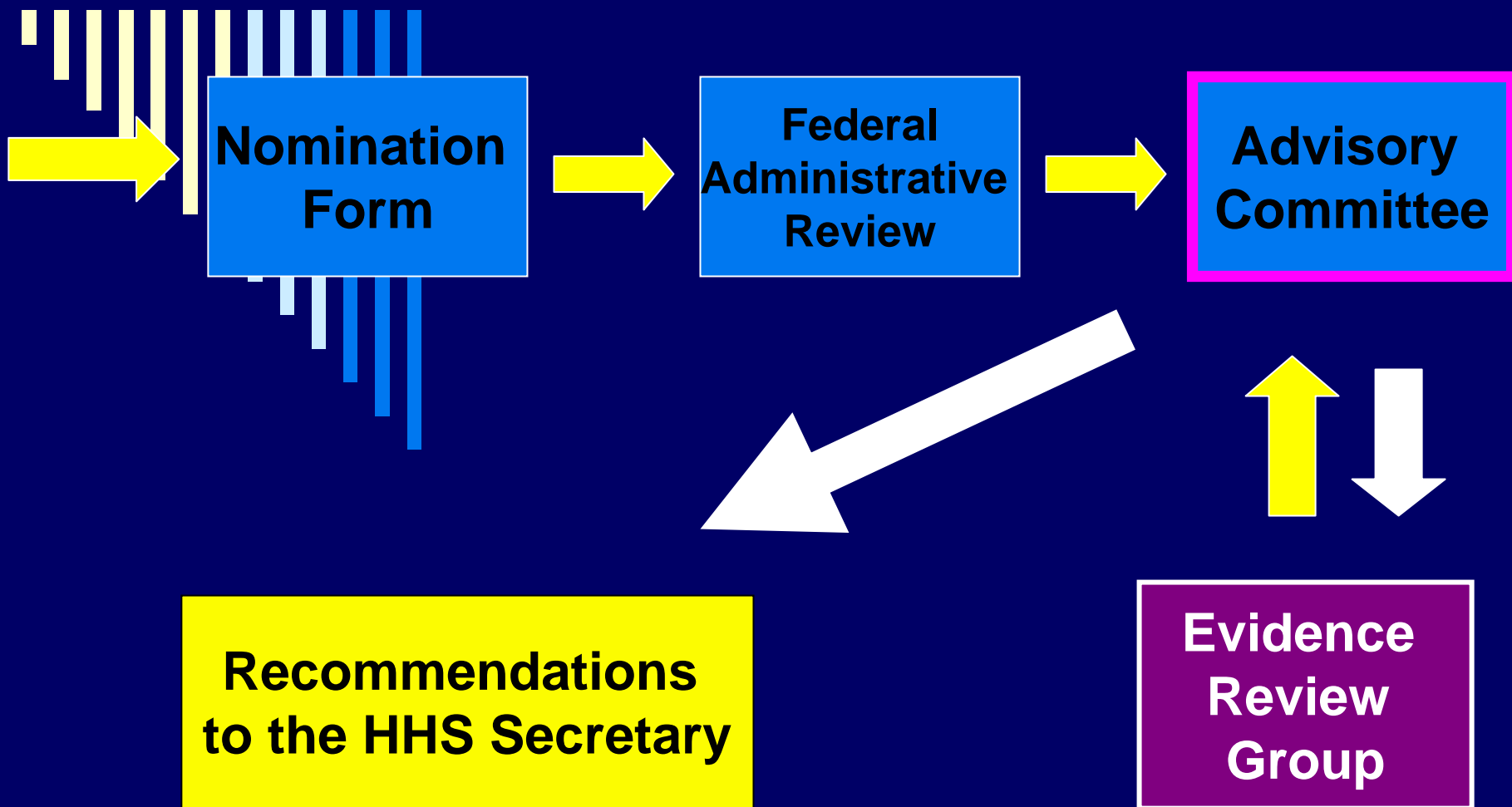
# The Nomination Process

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

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# Applying the Nomination Form

- Broader dissemination by MCHB
  - Definition of several terms
    - Severity
    - Accuracy (test)
    - Available (test)
    - Efficacy (treatment)
    - Urgency (treatment)
    - Risks (of screening and treatment)
    - Sensitivity and specificity floors
  - *Evidence Review Group will provide proposed definitions to committee and for public comment prior to next meeting*
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# 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
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# 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
    - Published evidence
    - Investigator findings (unpublished)
    - FDA trials database
    - Proprietary data
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# Paper on Pitfalls in Developing Evidence

- Main author: Alex Kemper, MD, MPH, MS
  - Based on October 2006 meeting and work done using Pompe disease as a prototype model
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# Evidence Review Group at the MGH Center for Child and Adolescent Health Policy

## □ Core staff

- Program director (policy, chronic conditions) James Perrin, MD
  - Project director (epidemiology/methods) Diane Romm, PhD, acting
  - Public health (from Mass. Dept of Public Health)
  - Consumer (Trish Mullaley, RN)
  - Cost/benefit analysis (Lisa Prosser, PhD)
  - (General) genetics (Marsha Browning, MD, MPH)
  - Health services research fellow (Ellen Lipstein, MD)
  - Methods and screening (Alex Kemper, MD, MPH, MS)
  - Consultant: Nancy Green, MD
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# Evidence Review Group External Support

- Assisted by members of ACHDGDNC and *ad hoc* expertise for specific disorder(s)
    - Clear conflict of interest policy
  - External advisory group – broader national representation and review
    - Ned Calonge, MD (Colo State Health Dept)
    - Robert Davis, MD, MPH (Ctr for Hlth Research, Kaiser Southeast)
    - Celia Kaye, MD, PhD (Univ of Colorado)
    - Ed McCabe, MD (UCLA)
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# Evidence Group Efforts

- Developing draft definitions of key terms for committee to review and public comment; finalize definitions based on comment prior to next meeting
  - Final template for evidence reviews determined – available at this meeting
  - Evidence group to carry out review(s) for spring meeting, based on Committee review of nominations
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# Evidence Review Template

- Background
    - Condition (prevalence, genetics, natural history, different forms of condition)
    - Rationale for current review (why now?)
  - Methods
    - Data sources
      - Human only; exclude case reports
      - How obtained and evaluated
    - Decision model and evidence questions
    - Data abstraction (and any new analyses)
    - Focus groups of experts (parents and investigators) as needed to estimate severity and burden
    - Screening and diagnostic testing
    - Treatment (risks, benefits; applicability to what condition groups)
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# Evidence Review Questions and Outcomes

- Key general questions
    - Natural history, including variations
    - Prevalence
    - Burden and severity
    - Methods of screening and diagnosis
    - Treatment effectiveness and variations (incl. Benefits and risks)
    - Costs of screening and treatment
  - 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 makes no recommendations
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