


Evidence-Based Reviews for Newborn Screening

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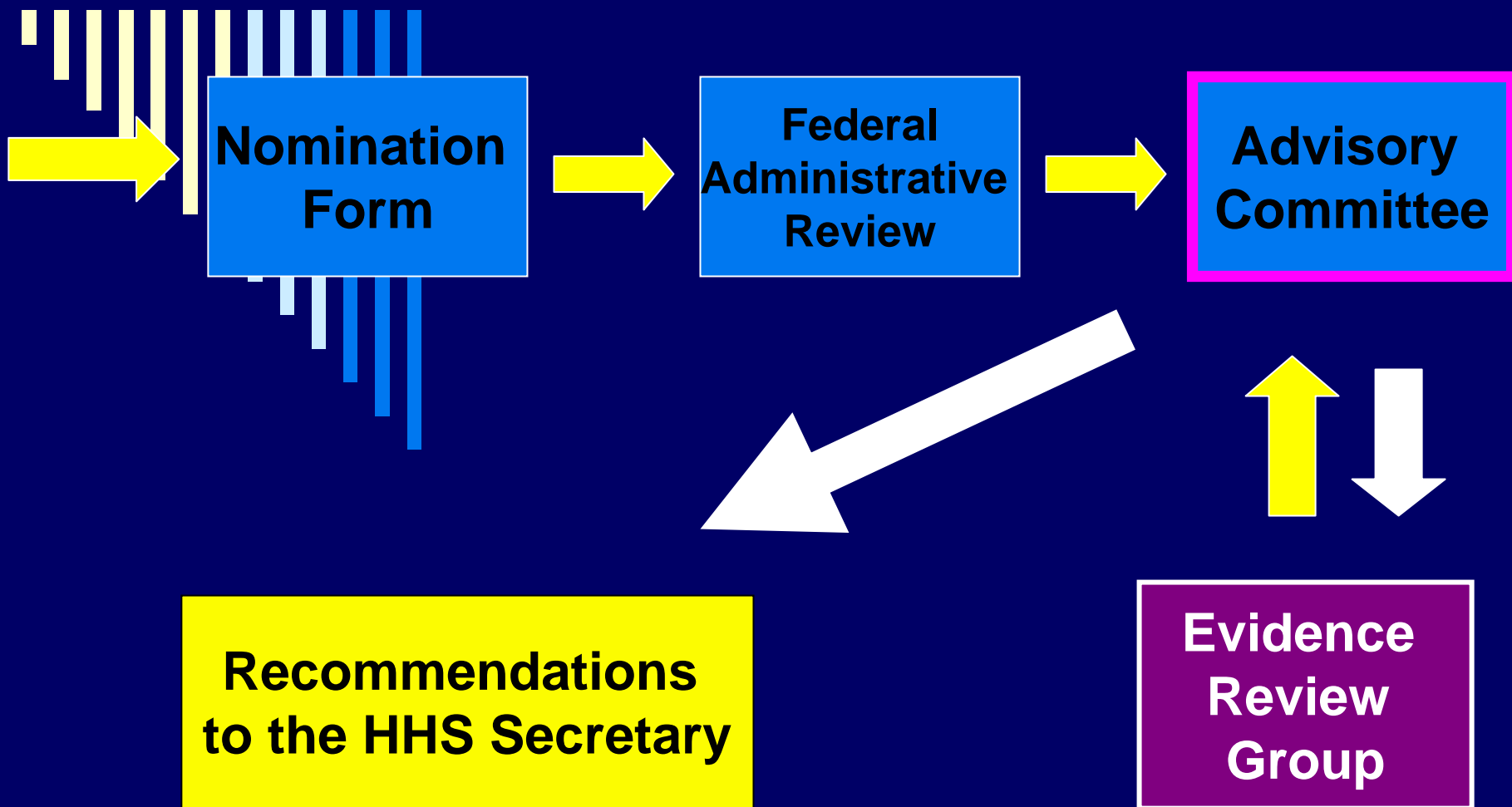
**MassGeneral Hospital
for Children**



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
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Nominations and Review





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
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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
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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

- FDA trials
 - Proprietary data
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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
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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
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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
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