Improving the Evidence Base for Pediatric Clinical Preventive Services

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Disclosures

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• The views expressed in this seminar do not necessarily reflect the views of the USPSTF or Group Health

• I have no financial disclosures relevant to this presentation
Overview

1. Evolution of evidence in pediatrics and clinical preventive services
2. Current state of the evidence
3. Challenges and opportunities to improve the evidence base for prevention in pediatrics
Evidence-Based Medicine

“…. is the conscientious, explicit and judicious use of current best evidence in making decisions about the care of individual patients”* 

Individual study vs. a ‘body of evidence

• Individual study quality:
  • Internal validity
    • Study design specific criteria
    • Minimization of bias and confounding
  • External validity
    • Generalizability
      Population
      Clinicians
      Setting
      Hierarchy of study design

* Sackett, DL. Evidence-based Medicine. Sem Perinatology 1997; 3-5
Evidence-Based Medicine

Evolution from consensus development statements to guidance based on systematic evidence reviews

Integrating Evidence Across Studies

- Standardized approaches to appraising a body of evidence
- Standards for meta-analytic methods from pooling of data across studies

BMJ 1995;310:1122
From Appraisal to Practice

Early pioneers in using SER’s and EBM-based clinical recommendations:

• Canadian Task Force on Preventive Health Care
• US Preventive Services Task Force
• Cochrane Collaborative

Institute of Medicine:

“Clinical Practice Guidelines We Can Trust”

Clinical Preventive Services

- Screening
- Behavioral interventions
- Chemo or immunoprophylaxis
- Mixed modality
The Evolution of Demand for Evidence

Priority and demand for evidence

1. Diseases of high severity, acuity and contagion
   - Studies of diagnostic tests
   - Treatment trials: reduce short-term morbidity and mortality
   - Prevention trials: reduce disease incidence

2. Diseases of high severity with short-term mortality risks

3. Chronic diseases with delayed consequences
Pediatrics and Evidence Demand

Two examples of high demand for evidence in children:

**Oncology**
- Acute lymphocytic leukemia

**Acute infectious diseases**
- Antibiotic therapy
- Passive and active immunization

[http://www.historyofvaccines.org/content/articles/scientific-method-vaccine-history](http://www.historyofvaccines.org/content/articles/scientific-method-vaccine-history)
The “New Morbidity” of Pediatrics

Acute morbidity and mortality replaced by attention to the ‘new’ morbidity in children

- Chronic illnesses
- Behavioral conditions
- Lifestyle risk factors (e.g. obesity)
- Behavioral and developmental pediatrics

New demand for treatment and prevention evidence

Evidence for Preventive Services in Children

USPSTF Recommendations Statements

Magnitude of benefit + Certainty of benefit

Grades
• A & B: Recommend routinely
• C: Recommend selectively
• D: Do not recommend

• I statement: Insufficient evidence

Evidence Appraisal Process

Magnitude net benefit = Benefit - Harms

Certainty = Body of evidence

Body of evidence includes:
- Internal validity
- External validity
- Heterogeneity
- Coherence

Size of evidence base
Are There Disparities in Evidence for Preventive Services?

- TOTAL: 128 Separate USPSTF Recommendations and I Statements
  - 38% are Insufficient Evidence statements
  - 62% are graded with A, B, C or D

- Of the 128 total statements, 36% (n=56) related to pediatrics
  - 21 are focused on infants and children (non-adolescents)
  - 35 address adolescents in the context of adult RS
  - 1 is focused only on adolescents (scoliosis screening)

- Absolute number of graded RS's is smaller, especially in young children
- Relative proportion of “I” statements similar between adults and children

www.uspreventiveservicestaskforce.org
Barriers to Achieving High Quality Evidence-based Pediatrics
Analytic Framework on Screening for a Disease: Pinpointing Evidence Gaps

From: Procedure manual, USPSTF, pg 20
Identifying Evidence Gaps in the Absence of Screening Trials

- Evidence for screening tools (KQ3)
- Evidence for treatment effectiveness (KQ 4, 5)
- Evidence for screening harms (KQ 7)
- Evidence for treatment harms (KQ 8)
- Evidence associating intermediate outcomes with health outcomes (KQ 6)
Classifying Evidence Gaps

- Closure of one or more gaps could convert an “I” statement to a letter grade

  OR

- Closure of gap(s) could enhance the magnitude of certainty for an existing recommendation

The USPSTF routinely identifies and reports evidence gaps
Special Challenges to Achieving Sufficient Evidence in Children

- ‘Macro’ barriers
  - Policymaker attention
  - Funder attention
  - Workforce
- Methodologic barriers
General Methodologic Barriers

- Low condition prevalence and statistical power
- Short term outcomes
- Lack of longitudinal studies bridging childhood to adulthood
- Generalizability of findings across all development stages
- Absence of modeling studies
- Health outcome metrics
- Heterogeneity of screening tools
- Heterogeneity of interventions
Analytic Framework on Screening for a Disease: Pinpointing Evidence Gaps

1. Early Detection of Target Condition
2. Persons at Risk
3. Screening
4. Treatment
5. Intermediate Outcome
6. Association
7. Adverse Effects of Screening
8. Adverse Effects of Treatment
9. Reduced Morbidity and/or Mortality
So What Now?

Should evidence standard for pediatrics match those for adults?

- IF YES, then:

  What investments are required to improve the availability of evidence?
Toward A Robust Evidence Base for Pediatric Preventive Services

• Investments in screening trials and cohort studies with adequate followup
• Develop infrastructure for multi-center prevention trials
• Align study designs to minimize heterogeneity
  • Screening tests
  • Treatment modalities
  • Outcome measurement
• Develop a set of robust epidemiologic reviews that associate intermediate outcomes with longer term outcomes.
The Role of Funders in Advancing Evidence

Enhancing Coordination Among the U.S. Preventive Services Task Force, Agency for Healthcare Research and Quality, and National Institutes of Health

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This paper focuses on the relationships among the U.S. Preventive Services Task Force (USPSTF); Agency for Healthcare Research and Quality (AHRQ); and NIH. After a brief description of the Task Force, AHRQ, NIH, and an example of how they interact, we describe the steps that have been taken recently by NIH to enhance their coordination. We also discuss several challenges that remain and consider potential remedies that NIH, AHRQ, and investigators can take to provide the USPSTF with the data it needs to make recommendations, particularly those pertaining to behavioral interventions. (Am J Prev Med 2015;49(3S2):S166–S173) Published by Elsevier Inc. on behalf of American Journal of Preventive Medicine. This is an open access article under the CC BY-NC-ND license (http://creativecommons.org/licenses/by-nc-nd/4.0/).
Questions

Send questions to prevention@mail.nih.gov

Or

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