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Improving the Evidence Base for Pediatric Clinical Preventive Services

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Disclosures



- Member and vice-chair, U.S. Preventive Services Task Force (USPSTF)
- 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:

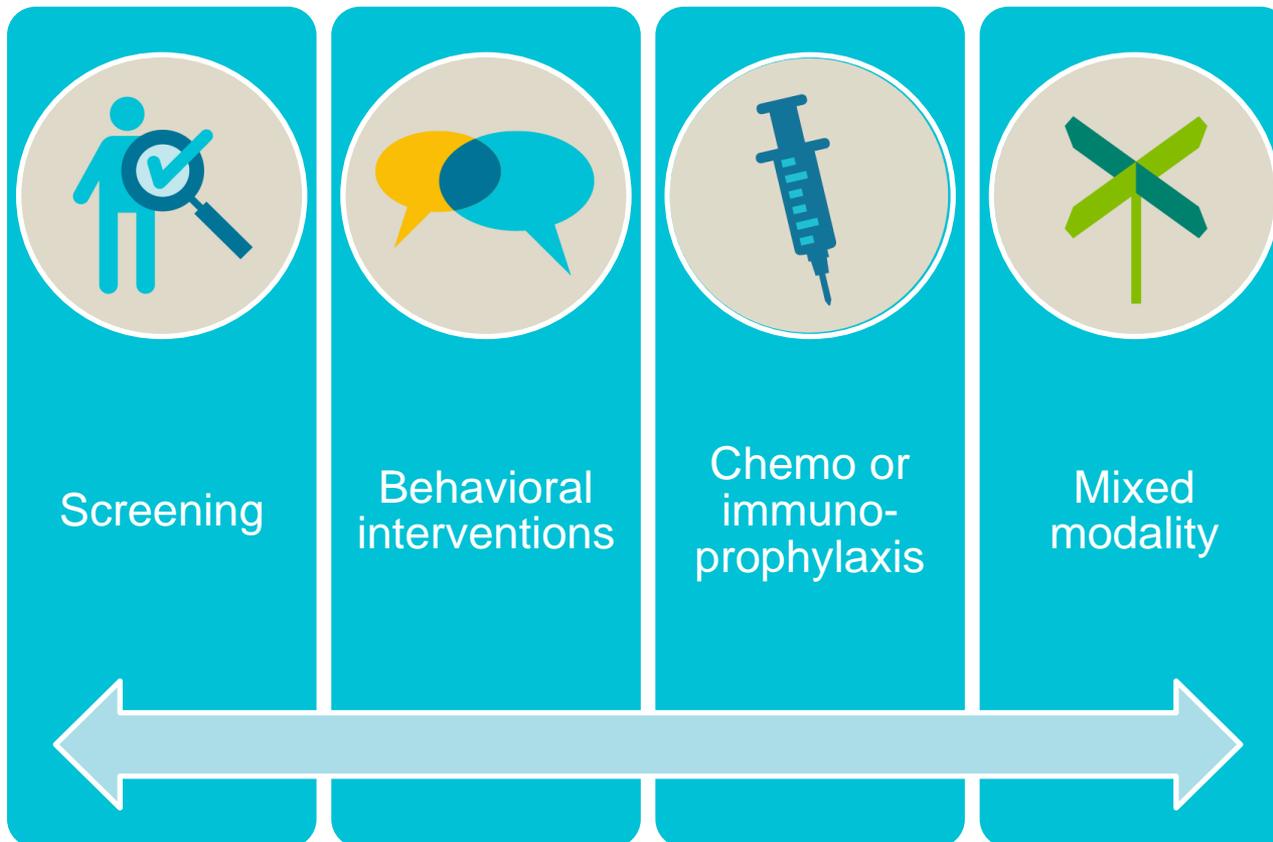
“Clinical Practice Guidelines We Can Trust”

IOM (Institute of Medicine). 2011. *Clinical Practice Guidelines We Can Trust*. Washington, DC: The National Academies Press





Clinical Preventive Services





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

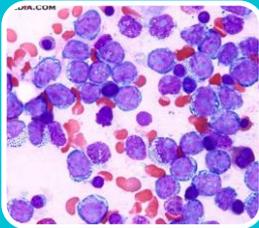
2.
Diseases of high severity with short-term mortality risks

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

https://en.wikipedia.org/wiki/Children%27s_Oncology_Group

<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)

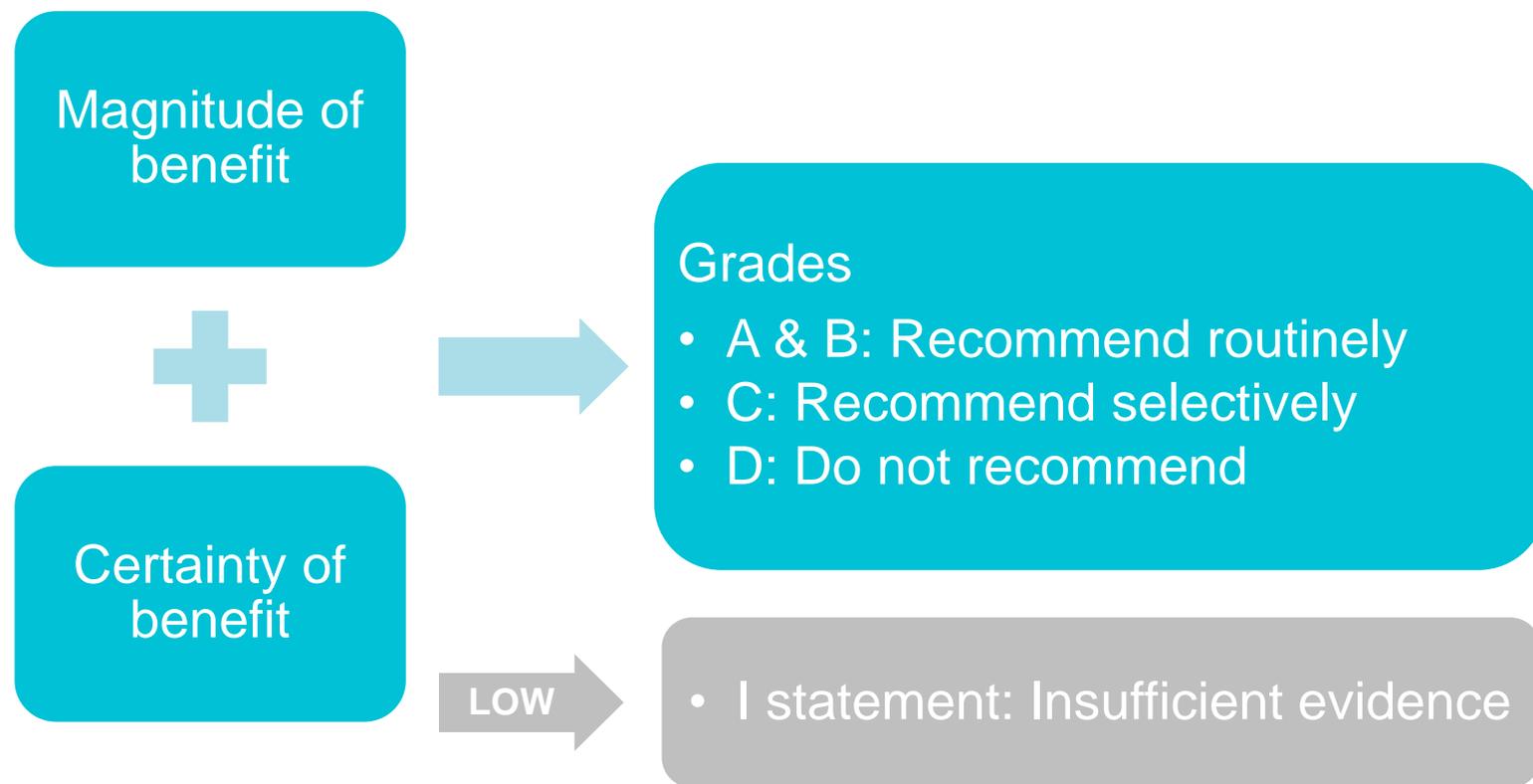
Behavioral and developmental pediatrics

New demand for treatment and prevention evidence



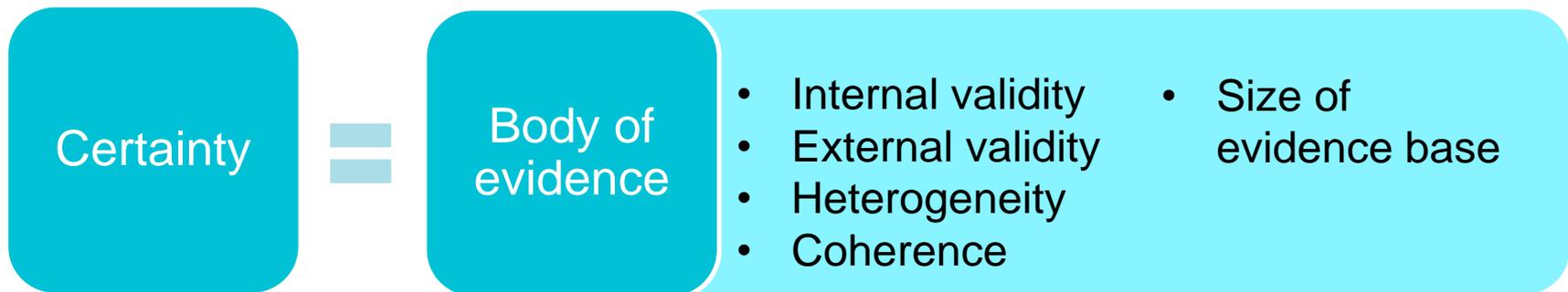
Evidence for Preventive Services in Children

USPSTF Recommendations Statements





Evidence Appraisal Process





Are There Disparities in Evidence for Preventive Services?

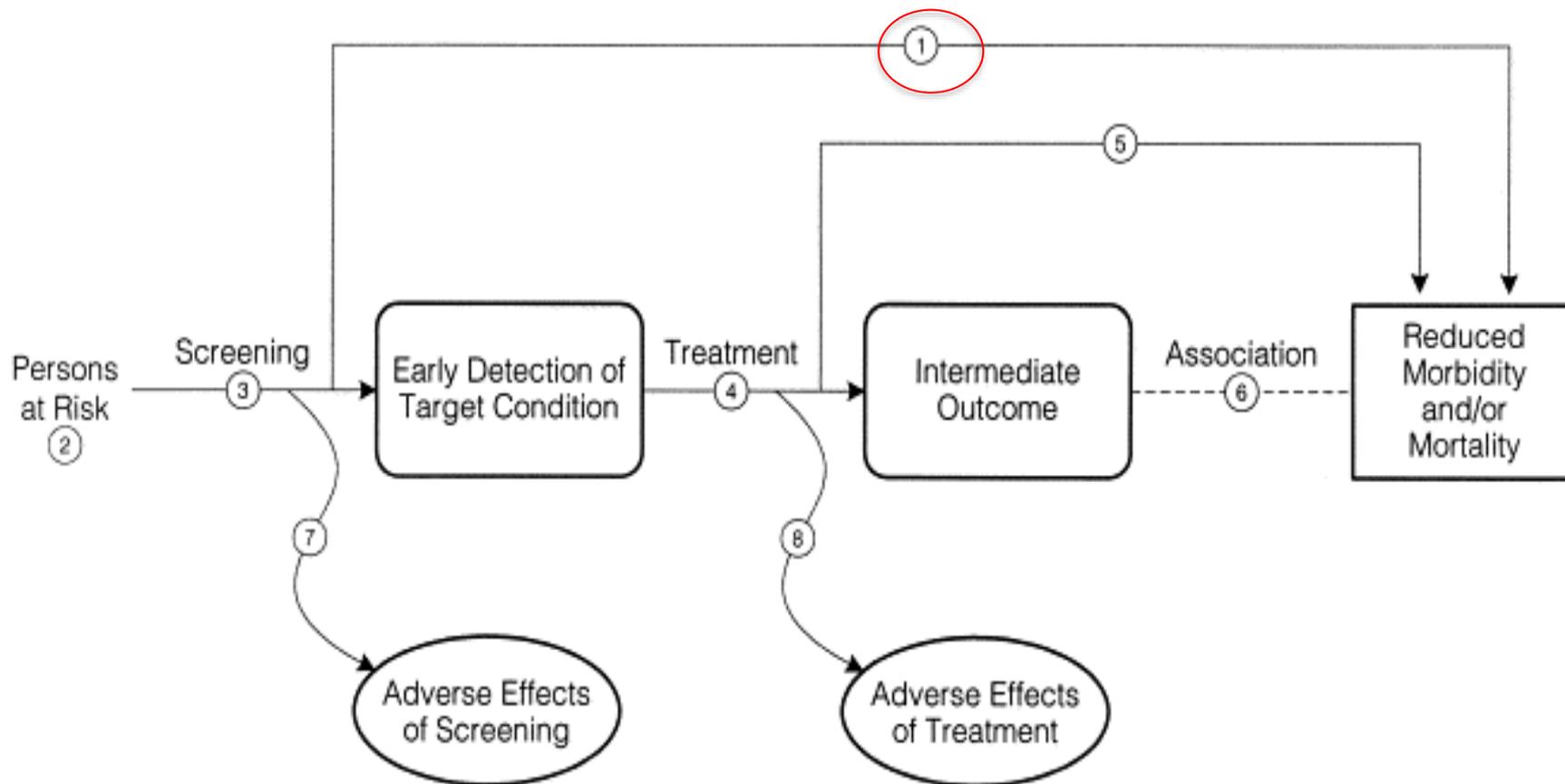
- TOTAL: 128 Separate USPSTF Recommendations and I Statements
 - 38% are Insufficient Evidence statements
 - 62% are graded with A,B,C or D
- Of the 128 total statements, 36% (n=56) related to pediatrics
 - 21 are focused on infants and children (non-adolescents)
 - 35 address adolescents in the context 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



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

SPECIAL ARTICLE

USPSTF Perspective on Evidence-Based Preventive Recommendations for Children

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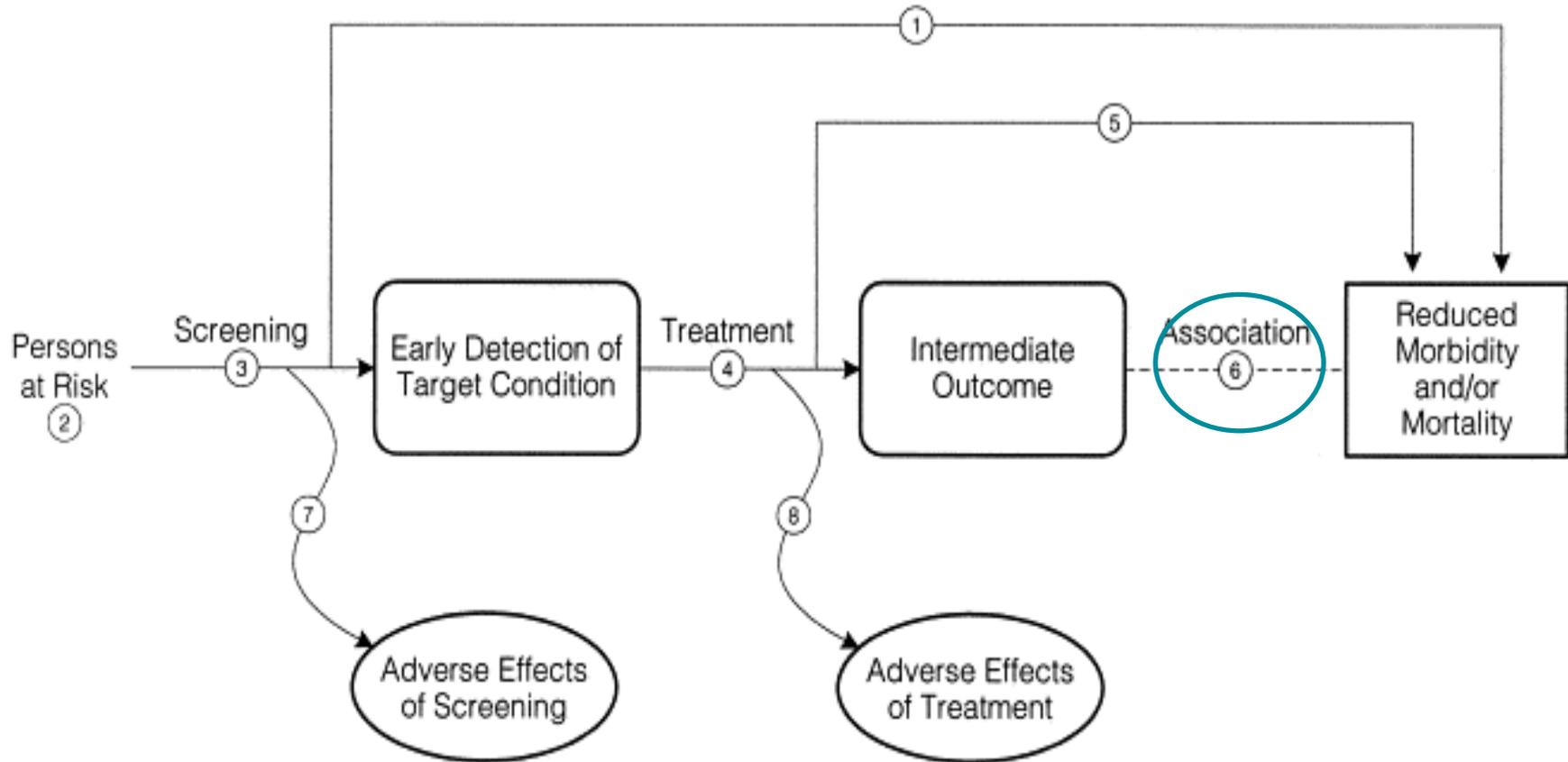
KEY WORDS
clinical preventive services, children, adolescents, evidence-based practice, primary care, guidelines, research

ABBREVIATIONS
AAP—Academy of Pediatrics
EBP—evidence-based practice
EPC—evidence-based practice center

abstract

The development and use of evidence-based recommendations for preventive care by primary care providers caring for children is an ongoing challenge. This issue is further complicated by the fact that a higher proportion of recommendations by the US Preventive Services Task Force (USPSTF) for pediatric preventive services in comparison with adult services have insufficient evidence to recommend for or against the service. One important root cause for this problem is the relative lack of high quality screening and counseling studies in pediatric primary care settings. The paucity of studies limits the development of additional evidence-based guidelines to enhance best practices for pediatric and adolescent conditions. In this article, we describe the following: (1) evidence-based primary care preventive services as a strategy for addressing important pediatric morbidities, (2) the process of making evidence-based screening recommendations by the USPSTF, (3) the current library of USPSTF recommendations for children and adolescents, and (4) factors influencing the use of USPSTF recommendations and other evidence-based guidelines by clinicians. Strategies to accelerate the implementation of evidence-based services and areas of need for future research to fill key gaps in evidence-based recommendations and guidelines are highlighted. *Pediatrics* 2012;130:e399–e407

Analytic Framework on Screening for a Disease: Pinpointing Evidence Gaps





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/>).



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