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Issue Brief

Evidence-Based Health Care for Children: What Are We Missing?

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ABSTRACT: With the enactment of comprehensive health reform, reimbursement for a variety of health care services will likely depend on evidence to support that provision. Understanding what constitutes “evidence” will have a profound effect on the range of clinical care provided. A too-narrow definition may have a considerable impact on pediatric care in particular: much of current child health care requires consideration of a broader body of evidence than is usually relied upon when developing clinical guidelines. This is especially true for care that addresses behavioral and developmental problems. The current standard for evaluating evidence uses study design as a proxy for the quality of evidence; it may therefore inadvertently exclude many important findings and fail to support further relevant research. The project described here yielded a new, broader framework for evaluating clinical practice, one that should be of value to both clinicians and policymakers.

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OVERVIEW

Over the past two decades, pediatric clinical practice in the United States has shifted from a predominant focus on disease and infection to one of health promotion and risk reduction. As a result of technological advances and new understandings in early human development,¹ child health promotion now underlies much of children’s medical care.² In the U.S., major threats to children’s health and well-being range from injury and abuse to obesity, developmental disability, and illicit drug use. Despite these substantial changes in childhood morbidity and its treatment, the tools used to gather evidence and measure the effects of health care interventions have not kept pace. Thus, the evidence supporting new, effective public-health-based approaches to child health promotion has not been given sufficient weight in the formulation of guidelines for care and reimbursement.

Unless the existing evidence framework is modernized and broadened, health care reform efforts that promote evidence-based care may inadvertently limit the use of effective interventions and may undermine advances in child

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health and health care, to the detriment of America's children.

Evidence-based medical care as we know it today came into being during the last decades of the 20th century as a method of evaluating the effects of short-term treatments for individual patients. The research and analytic techniques developed then have dramatically improved many areas of health care, from antibiotics for common infections to chemotherapy for difficult-to-treat cancers. By contrast, many child health promotion interventions do not involve medical treatment, per se, but aim to change the physical, social or emotional environment in which children live and learn. The effects of these changes may only become apparent many years in the future. As a result, child health promotion within pediatric medicine is considered evidence-informed, rather than fully evidence-driven.³ This issue brief describes a new framework for evaluating health care interventions that better suits the goals and methods of modern child health care.

EVIDENCE-BASED MEDICINE IN CONTEXT

The field of pediatrics began with specialized clinical services and research focused primarily on the care of newborn infants and on the prevention and treatment of childhood infections. These issues lent themselves well to study through the use of randomized controlled trials (RCTs). RCTs are designed to optimize causal inference for therapies directed at individual patients through scrupulous selection of subjects and carefully controlled experimental conditions. Owing in part to the tremendous advances that RCT has facilitated, these older threats to child health have receded in importance in the United States and other economically developed nations. Now, insults related to children's cognitive, social, and emotional development such as accidental injury, abuse, drug abuse, obesity, poor housing, and substandard education endanger more children than do infectious diseases. These conditions develop through complex interactions between biology and exposure to the physical and social environment,⁴ interactions that are not readily amenable to study through controlled interventions.

Today, health care is one of a number of factors that affect children's outcomes. Reflecting on the multifactorial determinants of children's health and well-being, a 2004 Institute of Medicine (IOM) report redefined child health as "the extent to which individual children or groups of children are able or enabled to develop and realize their potential; satisfy their needs; and develop the capacities to allow them to interact successfully with their biological, physical, and social environments."⁵

The breadth of this definition significantly expands both the potential inputs into children's health as well as its potential outcomes. It creates substantial challenges in demonstrating the relationships between them. There is an emerging consensus that RCTs cannot easily test the complex relationships implicit in most clinical interventions designed to promote health.⁶ And the cost and complexity of RCTs aimed at investigating typical children's health promotion interventions are complicated by these factors:

- Many child health promotion interventions are directed at the sites upon which children, by their very nature, are dependent: families, communities and schools. Evaluating the effectiveness of these indirect interventions through classical RCTs requires dramatically increased numbers of research subjects and substantially increased trial costs.
- The effects of interventions on children are frequently realized years later. This not only inflates the complexity and cost of research, but also risks rendering a study irrelevant by the time it is completed, as the conditions under study may have changed in the intervening decades.
- Childhood is characterized by change; singling out specific changes resulting from interventions can become a difficult task. Consequently, studies measuring dynamic effects in childhood require careful and complex assessments, and generally larger numbers of research subjects.

Exhibit 1. Comparison of Recommendations for Childhood Screening from the American Academy of Pediatrics (AAP) and the U.S. Preventive Services Task Force (USPSTF)

AAP/Bright Futures	USPSTF
Newborn hearing	Recommended (2008)
Child abuse and domestic violence	Insufficient evidence to recommend
Adolescent alcohol use	Insufficient evidence to recommend
Overweight	Insufficient evidence to recommend (revised to “B” in 2009)
Development in children under 5 years old	Insufficient evidence to recommend
Elevated blood lead level	Insufficient evidence to recommend

Note: The two organizations have different missions: the AAP seeks to provide comprehensive guidelines for clinical preventive care; the USPSTF reviews the published evidence for selected prevention activities, and considers health promotion for all ages.

Sources: American Academy of Pediatrics, *Bright Futures: Guidelines for Health Supervision of Infants, Children, and Adolescents, 3rd Edition* (Elk Grove Village, Ill.: AAP, 2007); and United States Preventive Services Task Force.

- Properly conducted RCTs require that subjects be as similar as possible, and receive precisely the same treatments. These same characteristics—homogeneous subjects, tightly controlled procedures, and additional resources provided to the clinical setting—may make the study results difficult to apply outside of a research setting.

These technical issues, combined with resource scarcity, have resulted in the successful completion of only relatively few high-quality randomized controlled trials of child health promotion in the clinical context. The United States Preventive Services Task Force (USPSTF), which is charged by the Department of Health and Human Services to make recommendations about preventive services that should be incorporated routinely into primary medical care, uses a transparent approach to assessing scientific evidence of effectiveness. Their published methods favor randomized trials. In fact, many of their intervention and outcome analyses include only evidence derived from RCTs.

As a result, the USPSTF has found insufficient evidence to recommend well-accepted components of child health promotion. The absence of USPSTF guidelines in many areas of child health has led children’s health professionals determined to provide high quality, comprehensive preventive care to rely upon recommendations from the American Academy of Pediatrics (AAP). The AAP recommendations are derived from

expert consensus based on more extensive sources of data and less rigorous standards. The AAP and the USPSTF have different missions and the advice they offer sometimes appears to conflict, as illustrated in Exhibit 1.

Given the practical limitations of applying RCTs to evaluate much of child health care, alternative approaches to obtaining, assembling, and evaluating scientific evidence are needed in order to promote the evidence-based practice of pediatrics. This issue brief describes the process and results of a collaborative effort to organize and evaluate new evidence standards for child health promotion. An organizing framework was collaboratively developed by a multidisciplinary group of leading practitioners, researchers, and policy-makers through an iterative, inclusive, consensus process that took place over the course of two years.

ENGAGING THOUGHT LEADERS IN REACHING CONSENSUS

This process began with an assessment of the literature available for guiding decisions in two key areas that had been reviewed by the USPSTF in 2005: obesity prevention and early childhood developmental assessment and intervention. In conducting our own broad review of the literature, we found 1,036 abstracts on obesity screening. Of those, only 18 were carried out in primary care settings and only one was an RCT; most described processes of care. Similarly among the 942

Exhibit 2. Baseline Opinions Regarding Evidence-for-Practice Decisions

Statement	Mean Rating (n=11)
The only important factor to consider when making a clinical practice decision is evidence of effectiveness.	2.36
Evidence of effectiveness is only one of a number of factors to consider when making a clinical practice decision.	4.55
Clinical practice decisions are based on many considerations. Evidence of effectiveness is only one factor and frequently it is not the most important.	2.45
With respect to evidence, clinical practice decisions should be based on the best evidence available.	4.82
Among types of evidence, when available, RCTs provide the strongest experimental evidence of causality.	4.00
RCTs should be the standard against which all research designs should be measured.	2.91
Anything short of an RCT is inadequate for making a truly informed decision.	1.73

1= Strongly disagree 2= Disagree 3= Neither agree/disagree 4= Agree 5= Strongly agree.
Source: Authors' original data.

abstracts for screening related to child development, growth and development, as well as child behavior and related topics, only one RCT was done in a primary care setting, and almost all outcomes were related to process measures.

Based on this work, a small, interview-based survey was designed and administered to 11 leading child health care researchers to assess their opinions regarding evidence-for-practice decisions. Exhibit 2

presents the survey results obtained at the initiation of the interview. In general, the respondents endorsed basing practice recommendations on a variety of factors and evidence, and tended to disagree with limiting evidence to that generated by RCTs.

There was general agreement that recommendations for preventive child health care could be based on a variety of expertise and evidence. However, to better examine the issues related to which questions called

Exhibit 3. Papers Commissioned and Presented for Year II Consensus Conference

Title	Author/s
Overview, History and Charge: Putting Our Discussion in Context	Robert D. Sege
Sufficiency of Evidence: When Is It Enough? ⁱ	Barbara Yawn
Child Health Promotion Interventions: Use of Decision Analysis to Determine When Further Study Is Worthwhile ⁱⁱ	Peter Neumann, Joshua Cohen
e-Delphi Results: Developing Evidence Standards for Child Health Promotion	Edward De Vos
Pediatric Preventive Services Assessment: Outcome Trajectory	Thomas Dewitt
Evidence Standards for Child Health Promotion: Community Level Outcomes ⁱⁱⁱ	Neal Halfon
Evidence Synthesis	Virginia Moyer

ⁱ B. Yawn, "Sufficiency of Evidence: When Is It Enough?" Paper presented at "Proceedings of the Development of Evidence Standards for Child Health Promotion: Preparing the Meal Conference (Rockville, Md.: 2007).

ⁱⁱ J. T. Cohen and P. J. Neumann, "Using Decision Analysis to Better Evaluate Pediatric Clinical Guidelines," *Health Affairs*, Sept./Oct. 2008 27(5):1467-75.

ⁱⁱⁱ N. Halfon and M. Hochstein, "Life Course Health Development: An Integrated Framework for Developing Health, Policy, and Research," *Milbank Quarterly*, 2002 80(3):433-79.

for which types of evidence, we enlisted the participation of a group of leaders in child health policy and practice. These included leadership from the American Academy of Pediatrics' Bright Futures initiative, members of the USPSTF, researchers involved in evidence-based reviews and policy, and individuals drawn from private and federal funding agencies. After a process that included interviews, structured working group meetings, as well as the examination of commissioned manuscripts and Internet-based electronic Delphi processes, the group moved from examining areas about which there was broad agreement and disagreement to the development of a new framework for understanding evidence in the field of child health promotion. A meeting of the participants was held in 2007 to further clarify the issues that are unique to child health promotion and to consider a framework to organize evidence for practice. Exhibit 3 presents topics considered at that meeting.

UNIQUE CONSIDERATIONS GUIDING EVIDENCE STANDARDS

At the core of the controversy concerning child health promotion in clinical care are two basic questions:

- What types of outcomes are the interventions under consideration designed to deliver?
- Based on risks and benefits, how certain would we need to be to make policies for implementation or reimbursement?

To address these questions, two types of factors that are extraordinarily important during childhood must be considered: 1) factors affecting the child's developmental trajectory, and, 2) factors related to the social ecology of childhood—that is, the social context that shapes children's health and the course of their development.

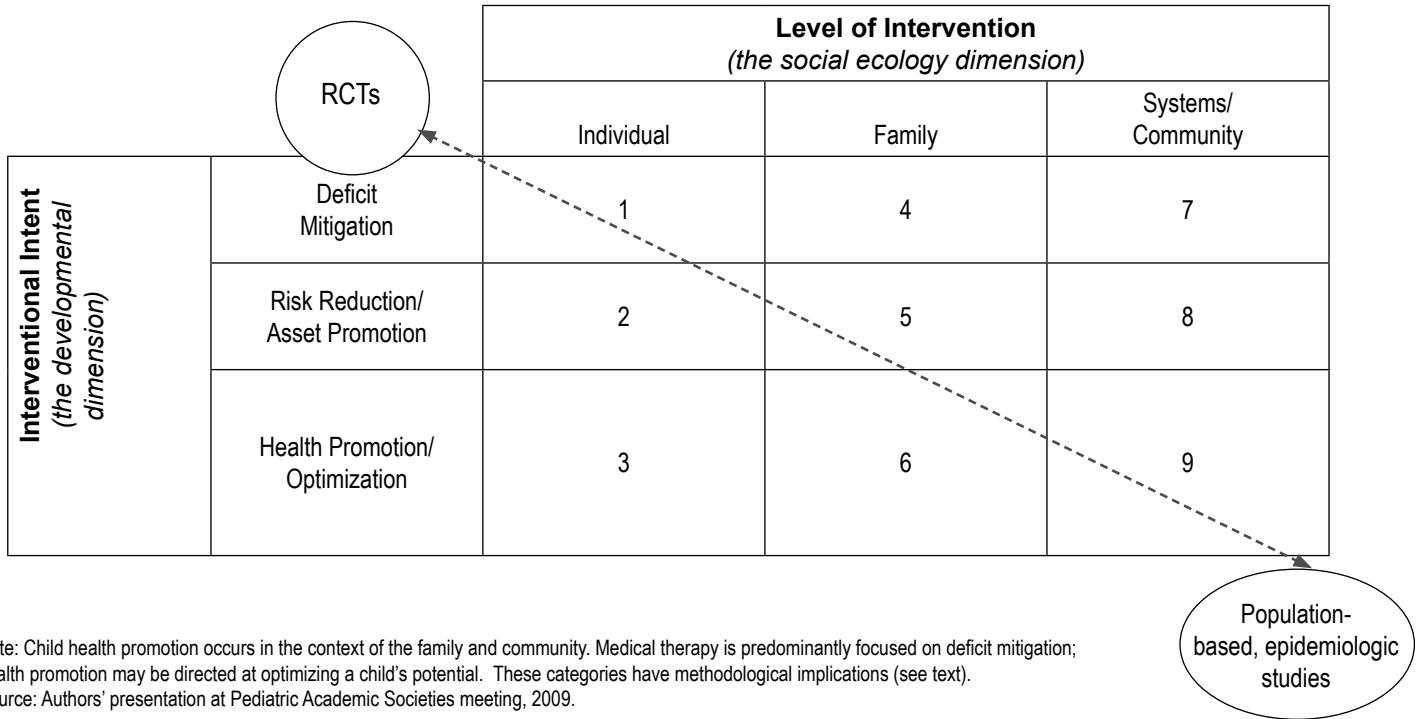
Children naturally progress through predictable developmental stages, marked by physical, emotional, social, and cognitive changes. The trajectory of their development, its rate and ultimate success in terms of adult health and functioning are influenced by a variety

of intrinsic biological and extrinsic environmental factors. These factors may be potentially damaging to the child's development (risk factors) or support that development (protective factors). Recent advances in functional brain imaging dramatically demonstrate the physical changes underlying child development, including the relationship between environmental factors and brain development, and suggest there may be particularly sensitive times for the attainment and optimization of skills,⁷ and times when adverse experiences have particularly detrimental effects on brain architecture.⁸ While secure parent–child attachment is associated with lifelong mental health, adverse child experiences may have severe, lifelong negative impacts.⁹ Thus, the outcomes that preventive health is trying to affect are ever-changing, more amenable to change at different stages of development, affected by both the child's genetic predispositions and acquired experience, and are often not fully realized until much later in life. In order to make a shorter-term assessment, there is increasing acceptance of using risk and protective factors as primary outcome measures.

The potential range of external factors affecting children's outcomes is enormous; they vary in terms of type, source, intensity, and timing. According to Bronfenbrenner's Ecological Model of Human Development,¹⁰ children grow in the context of family, school and community, and the larger society. Early childhood interventions typically focus on family or require its active participation. These interventions sometimes involve other community members or agencies (e.g., Head Start¹¹) as well. As children develop, the target of health interventions moves increasingly from the family toward the patient (e.g., HIV/STD prevention¹²), and the involved community is more often peers rather than service providers.

The two key aspects of children's health and well-being—developmental trajectories and social ecology—can be used to frame the relationship between desired outcomes and selected interventions. Many interventions will have multiple outcomes, which may vary in both their ecological and their developmental dimensions. Different study designs

Exhibit 4. The Evidence for Practice (E4P) Framework for Evaluation of Health Promotion Activities in the Clinical Setting



may be required to sensitively address different outcomes.

Exhibit 4 illustrates the interplay of these outcome dimensions. The rows relate to the primary intent of the intervention as it tracks to the child's developmental trajectory. That trajectory may be influenced by the presence of: 1) functional or biologic deficits; 2) risk factors; and/or 3) protective factors. Thus, interventions may be categorized as those that aim to reduce existing deficits; those that seek to reduce or modify risk factors; and those that increase protective factors.

The columns correspond to the major categories of influences that are part of the child's social environment. Some health promotion strategies focus primarily on the individual child; others target the family system or are intended to engage or alter larger systems, and involve community organizations and agencies or public policy. Comprehensive programs often intervene at multiple levels. Our framework provides a useful way to think about the various elements involved in preventive interventions. Exhibit 5 presents that framework we developed using some examples of existing

interventions; all of these interventions are designed to help prepare children to succeed in school.

Methodological Implications

The framework also provides guidance on when using RCTs to develop guidelines is likely to be more appropriate. Randomized controlled trials are designed, optimally, to measure interventions that focus on the individual and have the primary intent of mitigating a deficit (Exhibit 5, upper-left box). However, as we move away from this category, the suitability and feasibility of using an RCT to investigate the relationship between an intervention and an outcome tends to decline. Other methodological approaches, such as naturalistic observations, quasi-experiments, and program evaluation,¹³ become more practical and useful.

Health promotion interventions sometimes begin by screening populations of children in order to categorize individual children as being either unaffected, at risk for, or manifesting evidence of a health condition. In the public health model, interventions are described as universal (designed to address all children regardless of screening status), selective (targeting children at risk for a health problem), or indicated (applied to children

Exhibit 5. Framework for Categorizing Intervention Programs and Outcomes: The Evidence for Practice (E4P) Matrix

		Level of Intervention <i>(the social ecology dimension)</i>		
		Individual	Family	Systems/ Community
Interventional Intent <i>(the developmental dimension)</i>	Deficit Mitigation	Hearing, vision, cognitive screening	Adult literacy promotion	Immunization programs
	Risk Reduction	"Give a Child a Book"	Literacy-oriented family environment	HeadStart
	Health Promotion	Reach Out & Read	Reach Out & Read	Population-based, epidemiologic studies schools, libraries

Source: Authors' presentation at Pediatric Academic Societies meeting, 2009.

identified as having a condition). These categories coincide with traditional categories of preventive care: primary, secondary and tertiary. Protocols that involve screening will likely employ intervention strategies that vary according to the results of the screen. Thus, the choice of an appropriate study design to evaluate those interventions would also depend on those screening results. This distinction becomes especially important when evaluating evidence in order to make recommendations for individual care, as opposed to making recommendations for populations. The box on page 8 analyzes the example of lead screening. In this example, different frames of reference resulted in differing recommendations for practice.

THE QUESTION OF CERTAINTY: LINKING METHODS TO RECOMMENDATIONS

Most recommendations for clinical practice interventions rely only partially on the quality and quantity of evidence. Practice recommendations require balancing what can be concluded by reviewing available

evidence with the inevitable remaining uncertainty about questions that research has not and often will not address. Whether they are recommending national policy or considering the care of an individual child, as a practical matter, decision-makers must weigh the need for the intervention, the chance that it will succeed, and the expected, broadly defined risks, costs, and benefits of implementation. Generally, the greater the cost and the unanswered questions about an intervention, the more stringent will be the standards of evidence that are applied to making a recommendation. Alternatively, the lower the risk and cost and the greater the potential gain from the intervention, the less rigorous may be the research on which the decision is based. While some of these calculations take into account the priorities of the health care system, including the provider's and the patient's priorities, research studies provide estimates of overall risk and benefit, which may be included in drawing evidence-based conclusions.

The project participants felt that applying decision-analysis techniques could be helpful in weighing

Example: Lead Screening

There is little disagreement that lead is toxic to developing brains and that reducing the burden of childhood lead toxicity is beneficial. The Centers for Disease Control and Prevention, using traditional epidemiologic methods, has consistently recommended screening children for lead at intervals throughout childhood.ⁱ This type of intervention has resulted in substantial declines in overall childhood lead burden.ⁱⁱ The *Interventional Intent* would be “risk factor reduction” (reducing blood lead levels), and the outcome is measured at the “community” level.

The USPSTF asks a different question and arrives at to a different conclusion. Using their analytic framework, screening should be performed when there is therapy that will address the deficit or risk in the individual patient who screens positive. Their research determined that there were no published studies linking interventions to “improving neurodevelopmental outcomes in children with mild to moderately elevated blood lead levels.”ⁱⁱⁱ The USPSTF, therefore, found insufficient evidence to support lead screening as a method of addressing *individual patient deficits*.

Both of these recommendations are evidence-based; they simply addressed different issues and considered different evidence. Understood in this way, using the E4P (Evidence for Prevention) matrix as a guide, policymakers may consider evidence obtained through a variety of rigorous designs, not only RCTs, and make sound decisions regarding policy decisions.^{iv}

ⁱ “Recommendations for Blood Lead Screening of Young Children Enrolled in Medicaid: Targeting a Group at High Risk,” *Recommendations and Reports: Morbidity and Mortality Weekly Report*, Dec. 8, 2000 49(RR-14):1–13.

ⁱⁱ “Trends in Blood Lead Levels Among Children—Boston, Massachusetts, 1994–1999,” *Morbidity and Mortality Weekly Report*, May 4, 2001 50(17):337–39.

ⁱⁱⁱ “Trends in Blood Lead Levels Among Children—Boston, Massachusetts, 1994–1999,” *Journal of the American Medical Association*, May 23–30, 2001 285(20):2575–76.

^{iv} R. N. Shiffman, E. K. Marcuse, V. A. Moyer et al., “Toward Transparent Clinical Policies,” *Pediatrics*, March 2008 121(3):643–46.

the various factors that should be considered in making a recommendation for clinical care. Clinical policy recommendations, for example, are usually based on studies that compare proposed activities with the status quo, and only recommend change when there is a high likelihood that the new approach is superior. The decision-analytic framework does not put the status quo in this privileged position; new approaches that have a higher probability of success than current practice may be recommended.

Central to decision analysis is the issue of determining how much evidence is sufficient to recommend an intervention. The approach offers a systematic, transparent, and quantitative method for developing recommendations. It examines the costs and benefits of alternative actions, as well as the likelihood of both intended and adverse outcomes.¹⁴ This approach has major advantages in transparency, and can simultaneously evaluate and compare quite different interventions directed at similar outcomes. Decision analysis also highlights those areas where uncertainty may exist, and allows for analyses of the effects of this

uncertainty on the final, recommended approach.¹⁵ Further, by making explicit the instances where decision-makers disagree, or where weights change for different populations or over time, the implications of those recommendations are assessed directly.¹⁶

The Next Step: Assessing Research Quality and Validity

Although RCTs have long been known as the gold standard of scientific research, other study designs often prove more useful and are finding increasing acceptance in published literature. This is especially true when researchers are selecting a study design to test the effectiveness of a public health intervention. As described earlier, child health promotion strategies have certain characteristics that suggest, and in some cases require, the use of designs other than RCTs. For example, newer statistical techniques allow for greater use of observational studies and data. Propensity scores may be used to account for differences between treatment and control groups in situations where randomization is difficult or impossible.¹⁷ The Centers for

Exhibit 6. RE-AIM Framework

	Definition	Ecological Level
Reach	Participation rate; representativeness of participants	Individual
Effectiveness	Impact on health outcomes	Individual
Adoption	Participation rate; representativeness of settings	Organizational
Implementation	Consistency of delivery of intervention	Organizational
Maintenance	Individual: long-term effectiveness Organizational: sustainability	Individual and Organizational

Source: R. E. Glasgow, T. M. Vogt, and S. M. Boles, "Evaluating the Public Health Impact of Health Promotion Interventions: The RE-AIM Framework," *American Journal of Public Health*, Sept. 1999 89(9):1322–27.

Disease Control and Prevention’s Community Services Task Force maintains that the use of “before” and “after” trials with concurrent comparison groups, often termed “focal-local comparisons” offer strong evidence of effectiveness. Finally, Berwick has called for the use of quality improvement methodologies in studying effective health care improvements.¹⁸ These methods, drawn from manufacturing practices, have been successfully implemented in the child health promotion arena.¹⁹

In general, these alternative study designs seek to address the difficulties inherent in moving from well-conducted RCTs that tend to occur in tightly controlled experimental situations, to circumstances and populations more typical of clinical practice. Frameworks such as the RE-AIM model²⁰ (Reach, Effectiveness, Adoption, Implementation, and Maintenance) are able to evaluate the applicability—the external validity—of studies regardless of design. These frameworks facilitate the inclusion of epidemiological and other study designs in the evidence base on which everyday practice decisions are made (Exhibit 6). Explicitly assessing both the statistical design and the internal validity of a study, as well as its broader applicability, can replace the use of RCT study design as a proxy indicator of quality and validity.

SUMMARY AND RECOMMENDATIONS

Based on this work, we concluded that:

- Evidence-based standards favoring RCTs introduce hidden biases into policy formation;
- High-quality, useful research needs to optimize the conduct of the study itself, and include a thorough examination of the applicability outside the research environment; and
- The level of certainty needed to make recommendations for practice should vary in accordance with the likely risks, costs, and potential benefits.

These results have contributed to a wide discussion throughout pediatrics. In fact, these results have been presented and discussed at national meetings of health services researchers since 2006, at Academic Pediatric Association Meetings between 2007 and 2009, and at general national meetings of the American Academy of Pediatrics since 2007.

Focusing on shifting the methods by which evidence is understood is particularly appropriate for this moment in the history of American child health care: Health care reform and related methods of cost containment are being hotly debated; pediatrics is focused on interventions that maximize children’s development and promote their health and well-being not only during childhood but also over the course of children’s lives. In this context, it is important to reexamine the

standards of evidence that are used to determine effectiveness. Continued improvement of these methods is necessary to ensure that valuable services are included in the health care of American children.

Building upon a considerable literature concerning medical decision-making, practice-standard setting, and policymaking, as well as on input from a group of well-informed stakeholders, the Evidence for Prevention Project has found that the traditional reliance on randomized controlled trials to make preventive care recommendations systematically ignores important evidence. This reliance on RCTs thereby deprives practitioners of authoritative support for valuable clinical services. Study design should not be seen as a proxy for study quality. Well-designed studies employ a variety of methods designed to reduce bias and attain meaningful results. Moreover, the type and quality of evidence sufficient to recommend a particular intervention should depend on the intervention and its outcomes: low-risk, low-cost interventions do not require the same level of evidence as equally efficacious interventions of higher risk or cost.

Finally, in evaluating the relationship between evidence and the generation of guidelines, we make the following recommendations:

1. Policymakers should not rely solely on recommendations made using current USPSTF methods when determining covered preventative services for children: In practice, the USPSTF overwhelmingly relies on RCTs. Efficient study designs vary according to the characteristics of the intervention under consideration.
2. Policymakers should consider the cost, risk, and potential benefits of an intervention in assessing when there is sufficient evidence to make a recommendation. In general, low-cost, low-risk interventions may be justified even when the evidence is less solid; higher-cost/higher-risk interventions require stronger evidence.
3. Funding for child health research should be designed to better balance the need for internal validity with the need for results that can be generalized to typical clinical situations. Funding should be directed to those proposals that utilize the most efficient and appropriate research design for the intended outcomes. The process for awarding research funding should not use study design (e.g., RCT) as a proxy for study quality.
4. Peer-reviewed journals that act as gatekeepers for the dissemination of child health care research findings should encourage investigators to report on the external validity of their intervention research, just as they now require standardized reports of parameters associated with internal validity.

NOTES

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