PEDIATRICS PERSPECTIVES

Confronting the Need for Evidence Regarding Prevention

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When both of us entered pediatrics, we learned that screening dipstick urinalyses was a standard part of well-child care, even though data from at least the 1970s questioned its rationale based on the high false-positive rate and the identification of self-limited disease.[1] Screening seemed sensible and innocuous, and neither of us questioned it at the time. Although we were both aware of the debate about benefit, we do not recall hearing about the potential harms of dipstick screening. Over time, the evidence regarding the lack of benefit grew and the recommendation for screening urinalysis was limited to the 5-year-olds[2] and then eventually dropped.[3]

Here is the question: Should a screening test that seems effective be adopted into routine practice with the expectation that evidence would eventually affirm its use or remove it from clinical care? Do we "do first, ask later?" We all know practices in our communities that still routinely use screening urinalyses out of concern about missing a "case" regardless of the potential harm and questionable benefit. How much do we need to know about a preventive screening test before it is recommended as "standard of care?" When do we know enough to either start or stop a service provided to all? How well do we stop a preventive service after it is shown to be ineffective?

Preventive care services delivery is the cornerstone of pediatrics. From the newborn visit through adolescence, the American Academy of Pediatrics (AAP) and Bright Futures recommend 31 well-child care visits, each with specific discrete preventive services.[4] We are often asked by our trainees in clinic how we decide what services to provide within the limited available time. We were taught by skilled and talented clinicians based on their personal knowledge, experience, and expert opinion about what to do.

We now recognize that the standard should be higher. Pediatric preventive care services, like screening and counseling, are delivered to children who are asymptomatic and seemingly well and whose families often have no specific concerns about the target of the preventive care service. Although screening for some conditions and behaviors can lead to significant benefit, there is also the possibility of causing harm. Harms related to screening are often underrecognized, but can include physical

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harms (eg, diagnostic procedures after a false-positive screen, unnecessary treatments resulting from overdiagnosis), psychological harm (eg, anxiety, labeling), and induced financial strains for families (eg, missed work, co-pays for follow-up evaluation and care).[5] For example, screening urinalysis can commonly identify asymptomatic bacteriuria or orthostatic proteinuria, leading to a cascade of retesting and potential treatment without benefit to the child.

Understanding the relative benefits and harms of preventive services allows prioritization. The Bright Futures guidelines uses an "evidence-informed" process, combining elements of evidence-based medicine and expert opinion.[6] To what degree is the continuing use of expert opinion appropriate, especially given the lack of high-quality studies regarding the outcomes of many preventive services delivered in childhood? Will we always continue to rely on expert opinion or do we have an expectation that high-quality studies will be funded and conducted to replace expert opinion as our source of guidance?

The Institute of Medicine issued a report that serves as the foundation for clinical guidelines developed by many professional medical societies in adult medicine and could be a model for pediatrics.[7] A related approach specific for evaluation of preventive services is used by the US Preventive Services Task Force (USPSTF), of which we are both members. The USPSTF does not consider expert opinion in the final formulation of recommendations. It is also not enough to establish that a screening test can reliably identify a targeted condition. There must also be evidence that detection of the condition in an individual with no symptoms leads to better health outcomes. Preventive services can be recommended for routine care ("A" or "B" grades), recommended against ("D" grade), or identified for discretionary use based on the preference of the patient and family ("C" grade). When there is low certainty of net benefit, based on few or low-quality published studies, the USPSTF issues an "I" (insufficient evidence) statement. There is no room for expert opinion. An I statement is an explicit call for research to close the gaps that prevent a clear recommendation. Unfortunately, there are many I's in pediatrics.

These letter assignments are only part of the development of a full recommendation. For each preventive service, there is an accompanying comprehensive systematic evidence review and the USPSTF provides a detailed statement regarding how the decision was reached and the considerations for clinical practice and future research. Although expert opinion is not considered in the recommendations process, the USPSTF is dedicated to transparency in their methods and to ensuring that all relevant high-quality data are considered. Therefore, at the start of each preventive service evaluation, a draft research plan is released for public comment and public comment is also sought for the draft systematic evidence reviews and draft recommendation statements before they are finalized.

For many services we offer in pediatrics, the evidence base is incomplete. Should you screen for dyslipidemia in middle childhood, hypertension routinely during each visit starting in early childhood, or for autism in the second year of life? Although Bright Futures endorses each,[4] the USPSTF has assigned an I, based on the lack of evidence.[8] We understand the frustration of our colleagues regarding these differences and the challenge this creates in practice. However, identifying gaps is the only way to advance our field and ensure that we are providing the care that our patients and their families deserve. An I statement does not mean that there is no value to the preventive service or that the USPSTF is against its delivery. It is a tacit acknowledgment that we do not know if there is a net benefit to routinely providing the service to asymptomatic patients. Knowing that information can help clinicians to prioritize those activities for which a known net benefit exists.

Our concern is that many are complacent with reliance on expert opinion regarding the benefit of preventive services and do not advocate for the additional research necessary to ensure the benefit of pediatric primary care. We as pediatricians are trained to serve as advocates for our patients and families. The AAP is widely recognized as among the most respectable advocacy organizations because the balance of its major focus is on child health concerns, and not just professional practice. The AAP has often taken the lead in important but contentious debates, such as firearm safety. We believe that the AAP plays a critical role in child advocacy. The AAP has led research and safety advocacy efforts by supporting the Best Pharmaceuticals for Children Act and the Pediatric Research Equity Act, both of which were designed to increase the evidence base regarding benefits and harms of medications used in children, thereby reducing the need to prescribe "off-label" for children. Now that we are beginning to achieve parity for evidence regarding treatment, why would we demand less for preventive services?

We believe that the time has arrived for pediatricians and other child health providers to advocate for funding and executing research to achieve a higher-quality evidence base in pediatric preventive care. We need no less information on the optimal way to screen for

hypertension in children than we do for hypertension in adults.[9] We need more high-quality studies about the outcomes of early-identified cases of autism, including understanding the effectiveness of competing interventions. We should have as many trials and meta-analyses in prevention as we do for specialty treatment. We should not be afraid to question if 31 well-child visits leads to improved health outcomes compared with a lower or higher number. We should not be afraid to admit what we do not know, but then must also act to generate the missing knowledge. Pediatricians who are not involved in formal academics also can contribute to the understanding of the value of specific preventive services by participating in clinical trials conducted by practice-based research networks, such as the AAP's Pediatric Research in Office Settings network. As clinicians, we should adopt those preventive services known to be effective, studiously avoid those associated with harm, and seek to understand the evidence for each preventive service we offer in our busy practices.

E.B. White, the author of children's books, said, "The world is full of people who have never, since childhood, met an open doorway with an open mind." Let us together meet this particular open doorway with an open mind.

ABBREVIATIONS

AAP: American Academy of Pediatrics
USPSTF: US Preventive Services Task Force

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