Importance of Assessing Wellbeing for United States Preventive Services Task Force Recommendations

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Child health care providers spend a large portion of their time practicing preventive medicine. This includes screening, providing anticipatory guidance and counseling, and, less commonly, prescribing preventive medications.1 The US Preventive Services Task Force (USPSTF) makes recommendations on the basis of a systematic evaluation of scientific evidence that assesses the impact of clinical preventive services on child health, taking into account both the benefits and harms of preventive services.2 The USPSTF does not use expert opinion or clinical judgement when developing recommendations.

Because the USPSTF follows a strict evidence-based approach, it frequently finds insufficient evidence to support preventive services.3 The USPSTF issues "I statements" when it judges the scientific evidence underlying a preventive service to be insufficient to assess the overall balance of benefits and harms. An I statement does not signify that the preventive service is or is not beneficial but, rather, that the evidence base has gaps that preclude a definitive recommendation. Of the USPSTF’s 45 recommendations relevant to children and adolescents, nearly half are I statements, including screening for speech and language delay,4 autism spectrum disorder,5 and elevated blood lead levels6 and counseling or other interventions to prevent illicit or nonmedical drug use.7 Although each I statement has a unique set of reasons for why the relevant evidence is judged to be insufficient, the most common include a lack of data for the effect of the preventive service on ultimate health outcomes, including length or quality of life, and the inability to extrapolate evidence derived from research in symptomatic populations to screen-detected populations identified in primary care.

Overcoming these hurdles for developing recommendations for preventive services targeted to children and adolescents presents difficult challenges.8 Foremost, the low prevalence of most childhood conditions and a long time horizon between an intervention delivered in childhood and important health

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outcomes often measured in adulthood make assessing changes in health outcomes in trials of many preventive services impracticable.

There is a pathway out of the current limitations of the data on meaningful health outcomes for child and adolescent preventive services. The USPSTF defines health outcomes as “symptoms, functional levels, and conditions that patients can feel or experience and are defined by measures of physical or psychological wellbeing,” yet there are few published clinical trials that measure the effect of child-focused clinical preventive services on measures of subjective wellbeing, quality of life, or functioning successfully (eg, being ready for kindergarten, succeeding in school, graduating from high school or college, making friends, participating in community activities). These outcomes address the lived experiences of children and their families and, importantly, reflect an approach to assessing the meaningful implications of screening and intervention during childhood. Developing such measures and including them as prespecified outcomes in future trials represents an important, and potentially promising, way to address current evidence gaps. Although this pathway presents a number of challenges, it also offers new opportunities because it does not necessarily require unrealistically large trials or unfeasible follow-up periods that extend from early childhood to adulthood.

One example of a preventive topic that could benefit from this approach is the practice of screening children for speech and language delay. It is likely not feasible to determine if developmental screening in the infancy or early childhood impacts adult functional status. However, it would be possible to understand such screening’s impact on meaningful, nearer term assessments of subjective wellbeing: a child’s social aptitude or ability to make friends, their readiness for kindergarten, participation in age-appropriate activities, and the impact each of these has on the family. Of course, nearer-term changes in subjective wellbeing can have long-term implications for children and their families. In assessing health outcomes, the USPSTF looks specifically to outcomes that impact or can be inferred to impact the length or quality of life. Quality of life during one’s childhood is no less important than quality of life during adulthood. Trials could accurately measure these constructs and the changes to them in response to preventive interventions at appropriate time points over the life course. When combined with more traditional metrics of behavior and symptoms, these trials could conceivably move the USPSTF from an I statement to a specific evidence-based recommendation for a variety of its child health topics.

The shortage of subjective wellbeing outcomes in child-focused clinical trials, however, reflects not only their lack of use by researchers but also a lack of psychometrically sound measures from which to choose. In particular, there is a lack of measures that can be applied across health conditions or to domains relevant to multiple preventive health topics: for example, child social functioning, family functioning, or parenting stress. Consistent with the movement toward patient-reported outcome measures, a new generation of measures of subjective wellbeing needs to be developed and validated for children, adolescents and families. Although such measure development might take years, the investment would lead to better informed recommendations and improved delivery of preventive services.

Other issues complicate efforts to accomplish this important measurement task. A large share of preventive child health topics focus on infants and toddlers, and among this age group, measures of subjective wellbeing by necessity focus on family units, with parents or other caregivers as outcome reporters. The construct of subject wellbeing for young children needs to be conceptualized as a broader topic that focuses on the family. As children mature and become more capable of reporting their own wellbeing, instruments designed to capture such inherently subjective constructs will be variably sensitive, or insensitive, to change with intervention. This is particularly the case if the intervention being studied has not specifically been designed to impact the measurement construct. Furthermore, even if wellbeing could be measured accurately and precisely, self-perceived wellbeing changes over time. An intervention like providing a back brace for scoliosis may appear harmful in the near term, because of the pain and inconvenience of wearing a brace, but more beneficial later on. Assessing the balance of benefits and harms on subjective wellbeing, therefore, may be confounded by the timing of outcome assessment. As with the assessment of many other health outcomes, wellbeing should be measured repeatedly over time and integrated to understand the overall balance of change. In this sense, changes in wellbeing can be both important intermediate outcomes, with influences on a variety of health outcomes, as well as a being a specific long-term outcome.

Deploying valid and reliable measures of subjective wellbeing could help overcome the current
shortage of data on relevant health outcomes for preventive child health interventions. Moreover, such a strategy may also help overcome the complexities of extrapolating data from clinical populations to screen-detected ones. The inability of the USPSTF to make such an extrapolation in the treatment data for autism was largely responsible for the I statement on screening for autism spectrum disorder. In the case of autism spectrum disorder, changes in traditional outcome measures involve assessing symptom burdens. These changes are far easier to discriminate among highly symptomatic individuals whose condition was more likely to be detected on the basis of clinical suspicion or who receive care in a specialty setting. Such individuals are more likely to be enrolled in treatment trials for the simple reality that they are easier to recruit and more likely to generate positive results with respect to traditional, symptom-based outcomes. Conducting and powering trials according to outcomes that emphasize subjective wellbeing could create opportunities for studies that enroll populations with lower symptom burdens and allow investigators to measure constructs that may matter more to families than traditional dimensional symptom measures.

Practicing evidence-based preventive medicine is critical to the provision of good health care for children. Sadly, the reality is that we lack the kind of rigorous evidence that would allow us to assess the balance of benefits and harms even for common screening and counseling practices. Few would argue that this status quo is good for children or their families. The child health community should continue to encourage the development of large trials with long follow-up periods, but even if those were to happen, the standard of care for most topics will have changed during the decades of waiting for results. Developing measures of subjective wellbeing for children and families and deploying them at appropriate time intervals in prevention trials offers a promising, although admittedly challenging, pathway out of the child health evidence void.

ABBREVIATION
USPSTF: US Preventive Services Task Force

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