

Uso de servicios clínicos preventivos en niños y adolescentes

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Clinical Scenario

- A 2 year old boy comes in for the first time for a checkup. He has been well, and is up to date on his immunizations. He recently moved to Houston with his parents and two older siblings. He goes to preschool 3 mornings each week and his mother has no particular concerns about him.

What is recommended (in the US)?

- 13-29 well-child visits by the age of 18-21 years
- ***Bright Futures***: 80-100 individual suggestions for interventions at each visit
- AAP policy statements: 50+ interventions that “should be routinely included” in preventive health care

Services Recommended by Various Authorities (or Advocates)

- Screening
 - Lipid screening
 - TB screening
 - Immunizations
 - Developmental screening
 - Behavioral screening
 - Height/weight/BMI
 - Dental exam
 - Screen for physical abuse
 - Hearing screening
 - Lead screening
- Prophylaxis
 - Immunizations
 - Fluoride supplementation
- **Anticipatory Guidance**
 - Handwashing
 - Limit TV
 - Encourage physical activity
 - Car Seat
 - Smoke alarms
 - Swimming pool safety
 - Sunscreen
 - Smoke-free environment
 - Poison prevention
 - Gun safety
 - Dog safety
 - Bicycle helmets
 - No corporal punishment
 - Low fat dairy products
 - Toothbrushing
 - Toilet training

USPSTF Recommendations for 2 y.o.

- Screening
 - Visual Impairment
 - (Assumes newborn hearing, PKU and SSD screening)
- Prophylaxis
 - Immunizations
 - Fluoride
 - (Iron supplementation for high risk under 12 month old)

Why the difference?

- Who is the US Preventive Services Task Force?
- How do they decide on recommendations?
- How do they differ from other organizations (like the AAP or Bright Futures) regarding recommendations?
- Why does this matter to me?

US Preventive Services Task Force

- An independent panel of experts in primary care and prevention that systematically reviews the evidence of effectiveness and develops recommendations for clinical preventive services.
 - Government supported, but independent
- Provide **evidence-based** scientific reviews of preventive health services for use in **primary healthcare delivery settings** for patients without recognized signs or symptoms of target condition
- Age- and risk-factor specific recommendations for **routine** practice
- Recommendations include:
 - Screening tests
 - Counseling
 - Preventive medications

USPSTF “Ground Rules”

- You can't make someone who feels well feel better (but you can make them feel worse). Preventive services usually leverage a risk of *near term* harm against the possibility of *longer term* benefit
- Most individuals who receive a preventive service do not *personally* benefit from that service. (Think vaccines)
- All medical care – including preventive services – *can cause harm*

The USPSTF Steps:

- Assess the evidence across the analytic framework, synthesizing the assessment of each key question
 - ✓ Determine and judge the ***magnitude of both benefits and harms***: substantial, moderate, small, zero
 - ✓ Determine and judge the *balance* of benefits and harms: the ***magnitude of net benefit***: substantial, moderate, small, zero/negative
 - ✓ Judge the ***certainty*** of net benefit: low, moderate, high

Recommendation:
Balance of Benefits and Harms

Benefits – Harms = Net Benefit

State of the evidence for clinical preventive services in children

- For a few preventive services, there is evidence of effectiveness
 - Indirect evidence (that counseling changes behavior and behavior changes outcome) – skin cancer prevention, STI counseling, tobacco use counseling
 - Intensive, multifactorial programs including reinforcement over time more often result in behavior change – example: obesity
 - Intensive preschool vision screening by orthoptists resulted in less amblyopia and better vision (NNT=100)
 - Chlamydia and gonorrhea screening reduced incidence of pelvic inflammatory disease at 1 year
 - Fluoride supplementation and fluoride varnish in children under 5 years
 - Adolescent depression screening

State of the evidence

- For a few preventive services, evidence of *lack* of effectiveness
 - Nutrition education during routine health visits (27% vs. 28% anemic at follow up)
 - Counseling of mothers not to use pacifiers does not decrease pacifier use*
 - One RCT of 1 vs 2 newborn exams (n=9,712) found no difference between the two groups

*Good thing, too, since the AAP now recommends pacifier use!

State of the evidence

- For most recommended preventive services, lack of strong evidence of effectiveness
 - Screening for Chlamydia or gonorrhea in adolescent males
 - Routine HIV screening
 - Developmental screening
 - Autism screening
 - Speech and Language screening
 - Tuberculosis screening
 - Cholesterol/Lipid screening
 - Lead Screening
 - Growth monitoring
 - Blood pressure monitoring
 - Hip Dysplasia screening
 - Screening for physical and sexual abuse
 - Behavioral Risk assessment: alcohol and drug use, depression, suicide
 -
- You get the picture

Isn't an ounce of prevention worth a pound of cure?

- Vale mas una onza de prevención que una libra de curación?
- What harm could come from preventive services?

What harm could come from preventive services?

- Counseling:
 - Paradoxical increases in unhealthy behaviors
 - Harm to physician-patient relationship
 - Opportunity cost
- Screening:
 - False positives – people who previously thought of themselves as healthy are immediately less “well”
 - Incomplete understanding of disease
- Prophylaxis:
 - Iron poisoning
 - Rotavirus vaccine

Screening: the most common preventive service in pediatrics

- Unrecognized symptomatic disease
 - Depression, obesity, anemia, developmental delay, amblyopia, scoliosis, hip dysplasia, hearing loss
- Pre-symptomatic disease
 - Cancers, Newborn screening
- Risk factors for disease
 - Hypercholesterolemia, hypertension
- Predictors of poor health
 - Low health literacy, maternal depression, poverty

Why screen?

- Discovery of disease when it is more treatable
- If not treatable, then some other benefit
- Prevent progression *of* or *to* disease

Issues in Evaluating Screening

- Effectiveness of screening depends on
 - attributes of the test
 - effectiveness of early intervention
 - capacity of health care system
- By definition, screening is applied to persons with no signs or symptoms
 - Positive screen immediately worsens health status
- Hence, burden of proof for benefit is substantial

What are the six possible outcomes of screening?

with appreciation to Ned Calonge

THE AMERICAN BOARD of PEDIATRICS

Screening Outcome # 1

- Screening test negative...
 - but the patient *has the disease* - false negative - inappropriately reassured
 - Ignoring language delay because newborn hearing screen was normal

Screening Outcome # 2

- Screening test negative and the patient *does not* have the disease
 - True negative. No health benefit since patient does not have the disease
 - though patient reassured – is that always good?
- Is screening fatigue real?

Screening Outcome # 3

- Screening test positive...
 - But patient does not have disease
 - false positive – subject to risks/costs of further testing and anxiety
 - e.g. maternal serum testing for Down syndrome/Trisomy 18 is calibrated to label 5% of test results abnormal
 - Further tests may be invasive or dangerous

Screening Outcome # 4

- Screening test positive and patient does have disease...
 - but the patient is not destined to suffer morbidity or mortality related to the disease
 - treated unnecessarily
 - Example: 30-70% of men over 50 cancer cells in their prostates. Life time risk of death is 3%. How many of those detected by screening are treated for disease that would never have become clinically evident?

Screening Outcome # 5

- Test positive and the patient is destined to suffer morbidity or mortality related to the disease
 - but outcomes of treatment in asymptomatic stage are no different from treatment after symptoms are present
 - we simply lengthen the treatment time
 - e.g. what morbidity do we really prevent by screening for COPD with spirometry ?

Screening Outcome # 6

- Test positive
 - Patient destined to suffer morbidity or mortality related to the disease – and treatment in asymptomatic stage prevents complications that would develop if treatment not started until after symptoms are present
 - e.g. screening for colon cancer and treating in asymptomatic stage has clearly been shown to save lives

Keeping Score?

- For 5 of 6 outcomes, there can be NO health benefits to the patient
 - These 5 outcomes are not just costly – patients incur real harms of screening and treatment
- For 1 of 6 outcomes, there can be health benefits to the patient,
 - but no certainty that the benefits will exceed the harms of screening/treatment across the whole population

Mistakes in Screening: Neuroblastoma

- Meets the 1968 World Health Organization criteria
 - Important disease
 - Simple, inexpensive, sensitive test
 - Earlier treatment of clinical disease results in better survival
- BUT: Large scale trials do not support screening:
 - Expected increase in incidence of early disease
 - No decrease in incidence of late disease
 - No change in mortality
 - Possible increase in morbidity due to unnecessary treatment

Potential Harms and Costs of Behavioral Counseling

- The recommended intervention may itself be harmful
 - “Mr. Yuk” stickers
- Paradoxical increases in harmful behavior
 - Increased drinking in counseled youth
 - Reduced desire to quit in parents counseled to quit smoking
- Harm to the physician-patient relationship due to necessarily brief discussions of sensitive topics
 - screening and brief counseling for alcohol abuse (in a general practice) created more problems than it solved

Opportunity Costs of Behavioral Counseling

- 80-100 discrete counseling suggestions for each of the 29 well child visits recommended in Bright Futures
 - information about effectiveness or impact not provided
- 8 or more recommended counseling strategies for each adolescent visit recommended by GAPS
 - additional counseling based on individual patient needs
- Ineffective counseling strategies should not supplant effective preventive measures
- Using strategies of unknown effectiveness may cause loss of benefit

Potential harms of Prophylaxis:

- Iron poisoning
 - More common in families of treated children in a trial of iron therapy
- Rotavirus vaccine
 - Unanticipated increase in intussusception

Preventive interventions should be based on strong evidence of effectiveness.

- “Premature promotion of services that may be ineffective not only wastes time and money, but could also harm healthy patients, divert attention from more important issues, and undermine efforts to determine what really works”*
- “Establishment of ...programs for asymptomatic persons requires unequivocal scientific evidence”**

*Woolf and Atkins 2001

**Froom and Froom 1992

Availability and quality of research in child health

- Small sample size in many studies
 - Half of RCTs in one journal had $n < 20$
- Few available studies
 - Studies not done if intervention already tested in adults
 - Lack of funding for child health research
- Uneven study quality
- Mixed populations
 - Wheezing (mixed bronchiolitis and asthma)
 - DCCT (mixed adults and adolescents)

Methodological challenges in research on well child care

- How do we define the population?
- What are the interventions we want to study?
- What are the important outcomes?

Methodological challenges: Who is the population?

- Individual children
- Other children in the environment
 - Collateral benefit (or harm?)
- Families
 - Improved family function may also benefit the child
- Communities
 - Improved child health benefits communities

Methodological challenges: What are the interventions?

- Individual counseling interventions?
- Individual aspects of the physical exam as screening test?
- Is the global experience of caring and concern beneficial in itself?

Methodological challenges: What are the interventions?

- Is coordination of care (a “medical home”) beneficial?
- One intervention may affect many potential outcomes
 - Injury prevention counseling
 - Growth monitoring, developmental monitoring
 - Nutrition counseling
- “Bundling” makes sense conceptually, but is challenging to evaluate

Methodological challenges: What are the important outcomes?

- Rarity of dichotomous outcomes
- Rarity (fortunately) of mortality as an outcome
- Use of proxy measures
- Variability in how outcomes are measured
- Many important outcomes are a long time in coming
- Under what circumstances (if any) is early detection itself a valued outcome?

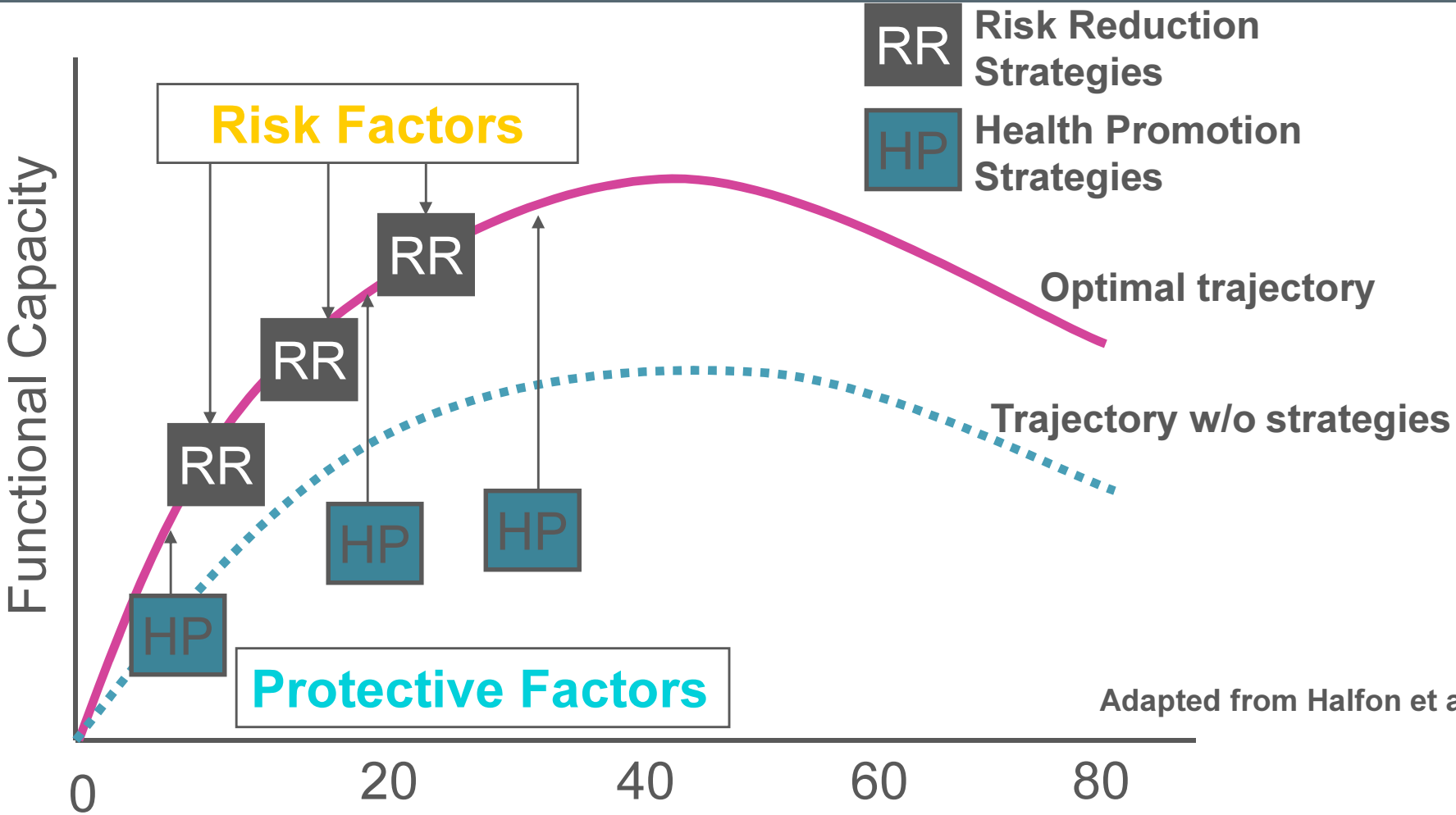
Methodological challenges: What are the important outcomes?

- What is the “right” health outcome
 - Maintenance of normal function
 - Need for assistance: glasses, hearing aid
 - Maximal potential for development
 - Family benefit

Methodological challenges: What are the important outcomes?

- What is “sufficient benefit”
 - Does small benefit in childhood represent large benefit in adulthood?
 - Healthy behaviors
 - Could the potential benefit vary with age?
 - Infant >toddler>child>adolescent

Developmental Trajectory



Age (years)

¡AVISO! Lack of evidence of effectiveness is not evidence of lack of effectiveness

- Some interventions are supported by very strong observational evidence
 - Back to sleep
- “Face validity” of some interventions
 - Pool fencing
- Potential for “collateral” benefits (and harms) is unknown
 - Lead screening

Moving beyond insufficient evidence

- Who should be providing individual preventive services?
 - health visitors vs. pediatricians
- How often are preventive visits needed?
 - 29 WCC visits (NB-21 yrs) recommended by AAP
 - 13 WCC visits (NB-18yrs) recommended by ICSI
- Is primary care the right setting for all of these interventions?
 - Schools, communities
 - Prenatal and early child home visits result in prevention of unwanted pregnancy and early childhood home visitation to reduce child abuse
- Can health systems provide proven services more efficiently?
- How will we know if these changes are effective?

Research Agenda

- Focus on implementation of interventions already proven
- Set research priorities for unproven interventions
- Involve the practicing community in research
- Systems research

If we are to be fair to children, we must attempt to protect them *from* medical care with the same passion as we attempt to protect them *with* medical care.

Mike LeFevre

Am Fam Physician. 2010 Sep 1;82(5):460-467.

Questions?

Thank you!
