Evidence Based Medicine: Scope and Limitations for Pediatrics

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Audience Survey(s)

- Fill out the survey you received (both sides)
- You only need to fill out ONE survey
- After you fill out one survey:
  - Trade yours with someone sitting to your right or to your left
  - Then trade again with someone sitting in front of or behind you
Disclosures

• Dr. Moyer is a member of the United States Preventive Services Task Force
  – This group makes evidence-based recommendations for preventives services for all Americans
• Dr. Moyer is a member of the AAP Steering Committee on Quality Improvement and Management
  – This group must approve all Clinical Practice Guidelines that come from the AAP
• Dr. Moyer established the TCH Evidence Based Clinical Decision Support project
  – This group develops guidelines for care, and develops tools to implement the guidelines in the integrated electronic record.
• Potential conflict: As a result of these affiliations, Dr. Moyer has a strong bias towards evidence-based care
“Well Child Care”

- Clinical Preventive Services
- Preventive interventions that are delivered to individual patients by (or under the direction of) the primary care provider
- Differentiated from community preventive services that occur outside of the context of individual patient care
¿Qué recomiendan los expertos?

• Si no está seguro, está bien adivinar.

• ¿Cuántas visitas del niño sano recomiendan los expertos entre el nacimiento y la edad adulta?

• ¿Cuántas pruebas de detección recomiendan los expertos en cada visita?

• ¿Cuántos temas de orientación anticipatoria (y preventiva) recomiendan los expertos deben ser discutidos en cada visita?
Audience Survey #1

• How many well child visits do experts recommend between birth and adulthood?  
  – __________

• How many screening tests do experts recommend at each visit?  
  – __________

• How many anticipatory guidance topics do experts recommend be discussed at each visit?  
  – __________
What is recommended?

• 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
Example
The 12 month visit

That's me

my Godson,
Thomas
Example: the 12-month visit

• Behavioral Counseling:
  – Nutrition:
    • iron rich diet, breast feeding, calcium intake
  – Injury prevention:
    • auto safety seats,
    • water heater temperature,
    • flame-resistant sleepwear,
    • poison prevention,
    • fire safety
Behavioral Counseling (cont’d)

• water safety,
• choking
• firearm storage,
• falls
• bicycle passenger safety
• protection from UV light,

– Mental Health

• Parental coping skills/stress reduction
Behavioral Counseling (cont’d)

– Dental:
  • nursing bottle caries,
  • wiping teeth with gauze,
  • dental referral

– Tobacco:
  • passive smoke exposure
Screening

• Interval History
• Physical Examination
  – Head circumference, Height, Weight, tooth eruption, heart murmur, hip dysplasia, evidence of neglect or abuse
• Developmental/Behavioral Assessment
  – Language development
• Vision screening
• Hearing screening
• Hemoglobin or Hematocrit
• Screen for TB, lead poisoning risk
Chemoprophylaxis and Immunizations

- Fluoride supplements
- Iron supplements
- Immunizations:
  - DTaP
  - Pneumococcal vaccine
  - Polio
  - Hepatitis B
  - Varicella
  - MMR
  - Influenza
What is this man doing?
He is drowning!

- The average pediatric preventive care visit is 15-17 minutes long
- Implementing only *proven* preventive interventions in primary care would take 7-8 hours/day, every day*

*Yarnall 2003*
Number of “Advice Directives” from the AAP

AAP “Advice Directives”

- 344 policies; 57 broadly relevant to clinical practice
- 192 discrete “advice directives” by 2002
  - “Parents should be advised that in 32 states, they have the authority to request that the DMV revoke the license of their minor child.”
  - “Pediatricians should…remind their patients’ families that if we do not buy or use entertainment media that are harmful to children, these media will no longer be produced.”
- Where is the evidence for all this advice?
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
Strong Evidence

- Data from studies least subject to bias
- For counseling (anticipatory guidance):
  - Observational evidence that change in a behavior leads to change in a health outcome
  - Data from clinical trials (randomized or quasi-randomized) demonstrating that counseling can change the behavior
- For screening
  - WHO criteria for a screening program AND
  - Data from clinical trials or concurrent cohort studies demonstrating that screening changes health outcomes
¿Cuál de estas están apoyadas por evidencia de buena calidad?

- La detección de niveles de plomo para mejorar resultados de desarrollo
  - Respaldada por evidencia de buena calidad
  - Evidencia de buena calidad no la apoya
  - No hay evidencia de buena calidad

- Etiquetas de Mr. Yuk para prevenir el envenenamiento
  - Respaldada por evidencia de buena calidad
  - Evidencia de buena calidad no la apoya
  - No hay evidencia de buena calidad
¿Cuál de estas están apoyadas por evidencia de buena calidad?

• La detección de infección por Clamidia para prevenir la enfermedad pélvica
  – Respaldada por evidencia de buena calidad
  – Evidencia de buena calidad no la apoya
  – No hay evidencia de buena calidad

• Asesorar a las nuevas madres el no usar chupetes para mejorar el éxito de la lactancia materna
  – Respaldada por evidencia de buena calidad
  – Evidencia de buena calidad no la apoya
  – No hay evidencia de buena calidad
How about the evidence?

- Screening for lead levels to improve developmental outcomes
- Providing Mr. Yuk stickers to prevent poisoning
- Screening for *Chlamydia* infection to prevent pelvic inflammatory disease
- Counseling new mothers not to use pacifiers to enhance breastfeeding success
How strong is the evidence for Well Child Care?

- We tabulated recommendations of selected major national groups
- Identified the most commonly recommended interventions
- Searched for systematic reviews and clinical trials
- Observational studies and consensus reports not considered
State of the evidence

• For a few preventive services, evidence of effectiveness
  – Indirect evidence (that counseling changes behavior and behavior changes outcome) – car seat and seat belt use, safe tap water temperature
  – Intensive, multifactorial programs including reinforcement over time more often result in behavior change – ex: physical activity
  – Intensive preschool vision screening by orthoptists resulted in less amblyopia and better vision (NNT=100)
  – Chlamydia screening reduced incidence of PID at 1 year
  – Folate supplementation prevents NTDs
State of the evidence

• For a few preventive services, evidence of lack of effectiveness
  – “Mr. Yuk” stickers increase attractiveness of poisons
  – Nutrition education during routine health visits (27% vs. 28% anemic at follow up)
  – Counseling of mothers not to use pacifiers*
  – Paradoxical increases in harmful behavior
    • Increased drinking in counseled youth
    • Reduced desire to quit in parents counseled to quit smoking
  – 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 gonorrhea in adolescent females
  • PAP smear in teens
  • Screening for Chlamydia or gonorrhea in adolescent males
  • HIV screening
  • Developmental screening
  • Speech and Language screening
  • Tuberculosis screening
  • Cholesterol/Lipid screening
  • Lead Screening
  • Growth monitoring
  • Blood pressure monitoring
  • Scoliosis 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?

- What harm could come from preventive services?
Why do we need evidence?

• Counseling:
  – Paradoxical increases in unhealthy behaviors
  – Harm to physician-patient relationship
  – Opportunity cost

• Screening:
  – False positives – patients (who previously thought of themselves as healthy) are immediately less “well”
  – Incomplete understanding of disease

• Prophylaxis:
  – Iron poisoning
  – Rotavirus vaccine
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
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
Mistakes in Screening: Neuroblastoma

• Meets the WHO criteria
  – Important disease
  – Simple, inexpensive, sensitive test
  – Earlier treatment of clinical disease results in better survival

• 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
Prophylaxis:

• Iron poisoning
  – More common in families of treated children in a trial of iron therapy

• Rotavirus vaccine
  – Unanticipated increase in intussusception
Recommendations Matter

• Broadening of primary physicians’ perceived obligations
• Used as basis for evaluation
  – Quality Assurance
  – Pay for Performance
• Used as basis for legislative mandates
  – The state of Texas used to require the use of the Denver II, a very poor screening test
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 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

- **Risk Factors**
  - Risk Reduction Strategies (RR)
  - Health Promotion Strategies (HP)

- **Protective Factors**
  - HP

- **Trajectory**
  - Optimal trajectory
  - Trajectory w/o strategies

Adapted from Halfon et al. 2000
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
What is a pediatrician to do?

• Prioritize interventions:
  – Use an informed approach to choice of interventions in individual practice
  – Insist on knowing the evidence before following recommendations

• Participate in research
  – practice-based networks

• Pay attention to the needs and wants of patients and families
  – address patient concerns first
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“Are you just pissing and moaning, or can you verify what you’re saying with data?”
Importance of evidence from trials

• Observational evidence is subject to bias
  – before after studies confounded by temporal trends in outcome, intra class correlations
  – non random allocation of intervention and control sites confounded by factors related to site that volunteered for the intervention; also intra class correlations
  – comparison of participants to non participants confounded by compliance bias
Analysis of the same data: observational vs experimental

• 2 clinical trials of interventions to increase breast feeding
  – supplemental formula
  – pacifier use
• Data analyzed by intention to treat – comparing intervention to control groups
• Data re-analyzed by observation – comparing outcomes between formula or pacifier users and non-users
Formula supplementation: observational vs experimental

- **Observational analysis:**
  - 100% vs 0% supplemented
  - 49.1% vs 23.7% weaned at 9 weeks
  - RR = 2.1
  - Confirms estimates from prior observational studies

- **Experimental analysis:**
  - 62% vs 15% were supplemented
  - 45.3% vs 45.9% weaned at 9 weeks
  - RR = 1.0
  - No effect of formula supplementation
Pacifier Use: observational vs experimental

• Observational analysis:
  – 100% vs 0 % used pacifier daily
  – 25%% vs 12.9% weaned at 9 weeks
  – RR=1.9
  – Confirms estimates from prior studies

• Experimental analysis:
  – 84% vs 61.4% used pacifier daily (p<.02)
  – 18.9% vs 18.3% weaned at 9 weeks
  – RR=1.0
  – No effect of decreasing pacifier use