

Component 2: Evidence- Based Medicine

Unit 5: Evidence-Based Practice Lecture 5

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Using EBM to assess questions about harm or etiology

- Question is not whether someone with exposure to agent gets ill, but rather those with illness have higher rate or amount of exposure
- Ideally assessed by RCT but this may be impractical or unethical
- Next best evidence comes from observational studies, which have limitations

Examples of questions to answer about harm

- Do silicone breast implants cause autoimmune diseases, such as lupus? (Gabriel, 1994)
 - Women with silicone breast implants developed connective tissue diseases and arthritis but at no higher rate than those without them
- Do anti-obesity drugs (e.g., fenfluramine and phentermine, also known as fen-phen) cause heart valve abnormalities? (Gardin, 2000)
 - Those who used these drugs developed certain heart valve abnormalities at a higher rate than those who did not

Hierarchy of evidence for harm

- Randomized controlled trial
- Cohort study
- Case control study
- Case series/report

Evidence and its limits

- Randomized controlled trial
 - Ideal, but often cannot be done or would be unethical to do so
- Cohort study
 - Prospective study without randomization
 - Is particularly useful when poor outcomes are rare and huge sample size would be required, e.g., upper gastrointestinal hemorrhage with NSAIDs
 - Are problematic when groups are really not similar, e.g., people who take NSAIDs may be sicker or otherwise different than those who do not

Evidence and its limits (cont.)

- Case control study
 - Most common form of observational study
 - Retrospectively identify cases of diseases and match to otherwise similar controls, looking to see if different rate or amount of exposure
 - Can be useful when condition is very rare or has long development time
 - Classic case was demonstration that DES causes vaginal cancer (reviewed in Swan, 2000)

Evidence and its limits (cont.)

- Case control study (cont.)
 - Problem is when controls create spurious association, e.g.,
 - Coffee drinking associated with pancreatic cancer (MacMahon, 1981), but controls were patients with other GI diseases whose symptoms were exacerbated by coffee (so they drank less)
 - Differences were not present when other appropriate controls were used (Zheng, 1993)

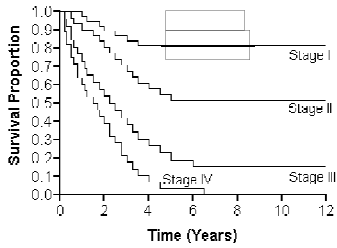
Evidence and its limits (cont.)

- Case series/report
 - No comparison group
 - Famous example was Bendectin for nausea in pregnancy, where adverse publicity led to removal from market of safe and effective treatment
 - Actually was combination of two agents, both of which were effective and neither of which were harmful (Magee, 2002)

“Pure” prognosis studies are rare

- Prognosis is “natural history” of disease
- But very little “history” is “natural” in modern era with our abundance of diagnostic tests, interventions, harmful agents, etc.
- Many studies measure prognosis after a test or intervention

Prognosis usually measured by a survival curve (Dunn, 2002)



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Example studies of prognosis

- Extremely pre-term birth (Marlow, 2005)
 - Followed cohort of 241 children from UK and Ireland born at 25 or fewer weeks gestation
 - Compared with 160 classmates born at full-term
 - 41% of pre-term children had “serious impairment” on cognitive assessment compared with 1.3% in control group
- Untreated early, localized prostate cancer (Johansson, 2004)
 - 223 men followed from 1977-1984
 - 17% developed generalized disease
 - 16% died of disease

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