Practice-Based Research Networks: Comments

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A practice-based research network (PBRN) is a group of practices devoted principally to the care of patients and affiliated for the purpose of examining the health care processes that occur in practices. PBRNs are characterized by an organizational framework that transcends a single practice or study. This provides a “laboratory” for studying broad populations of patients and care-providers in community-based settings. (Wikipedia)
Purpose of PBRNs

• “examining the health care processes that occur in practice”
  – Epidemiology: course of illness studies
  – Evaluation of medical tests and diagnoses
  – Improvements to care delivery (RCTs?)
  – Effectiveness trials (RCTs)
Problems

• Research questions must be of direct interest and importance to patients and clinicians in practice.
• Research designs must reflect strategies that can be (or are) used in clinical practice, not what is convenient for researchers.
Population

- “practices devoted principally to the care of patients”
  - “...strategies that integrate trials into routine clinical care” (Lauer)
  - Compromise between the Scientific ethic and the Clinical ethic (Feinstein).
Problems

• RCTs have generally been designed, conducted and analyzed by specialists in RCTs.
  – Simon: “which aspects of clinical trials reflect scientific necessity and which simply reflect tradition.”

• Clinicians in practice must buy into the idea of a research protocol.
  – Control/comparison treatment, randomization, “blinding”, etc.
Control/Comparison Groups in RCTs

• Necessity!
• Seldom, if ever, a Placebo Control group.
  – Does not usually reflect a decision a clinicians would make.
  – Instead: TAU, Standard of Care.
• Randomization the easiest and safest option.
• If not possible?
  – Propensity Score Analysis etc.
• Randomization of sites/clinics/clinicians instead of patients.
“Blinding”

• The issue is not “blinding” ‘per se’, but absence of bias in assessing outcome. (Simon)
  – RDoC Measures?
  – CATIE-type outcome?
  – Clinical Preference scores?
    • Functional Measures
Generalizability of Conclusions

• Sample more representative of the populations clinicians are called upon to treat.
  – Heterogeneity among patients, across sites.
  – Moderators (Personalized medicine).

• The multi-site advantage
  – Heterogeneity of effects over sites.
  – Necessity for independent replication/validation less urgent.
  – Greater understanding of site differences.
Outcomes

• Clinical/functional endpoints
  – NOT surrogate endpoints (Lauer).
  – NOT outcome measures of no interest to patients, clinicians.

• Integrating consideration of harms and benefits—just as clinicians do.

• Longitudinal Follow-up—just as clinicians do.
• Cross-over designs? N-of-1 designs? (Dubois)
• Longitudinal follow-up—intensive designs!
• Staged Treatment designs (STAR-D)!
  – Maintenance studies
  – Branched designs
  – Sequential treatments designs
Analytic Options

• Powerful, cost-effective designs/analyses
  – Analyzing the time course of response, rather than any single univariate outcome measure
Organizational Framework

• Duties of the “Steering Committee”
  – To design the study
  – To monitor the implementation of the design at multiple sites (fidelity)
  – To cumulate and clean the data
  – To do the statistical analyses
  – To report the findings.

• Must involve BOTH RCT expertise AND clinicians from each site.
Is this Worth Doing?

- If done, results are MORE valuable than those of traditional RCTs.
- If done, costs of doing studies LOWER than those of traditional RCTs.
- If done, results likely to MORE RADIDLY benefit patients.
Major Problem:

• “This is not the way we’ve always done it!”
• Are clinical researchers going to be willing to compromise for a common goal?