RCT vs. Observational Studies
A View from a Healthplan

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Randomized Controlled Trials
• Established the “efficacy” and “safety” of the treatment modality
  ✓ demonstrates that the drugs can produce the expected clinical effect
  ✓ demonstrates safety
  ✓ demonstrates adverse effects
  ✓ demonstrates possible risks
• Limitations of Randomized Controlled Trials
  ✓ application to a “real world” clinical practice setting
    i.e. population demographics, patient restrictions/monitoring, co-morbidity
  ✓ Limited to only “non-rare” diseases i.e. limited population size
  ✓ Limited duration of the trail

Observational Studies
• Studies that demonstrate a causal relationship between a treatment and an effect on a non-controlled population
  ✓ Represents a “real world” practice setting
    i.e. comorbidity
  ✓ Applicable to less prevalent disease/conditions
  ✓ Longer term studies i.e. safety evaluations
  ✓ Usually conducted by independent organizations
  ✓ May actually lead to more breakthrough treatment
• Limitations of Observational Studies
  ✓ Difficult to avoid or assess bias
    i.e. not an easily developed skill within healthplans
  ✓ Conclusions are not always easily applicable across a generalize population
    i.e. population demographics may not completely match

The CareFirst Population

Conclusions
• RCT and Observational Studies are needed to comprehensively treat any given population
  ✓ RTC provides the basis for the treatment
  ✓ Observational studies allows for adaptation to the population
• Observational studies can be thought of as “on-going” to allow for constant refinement to the population
• While hierarchies exist, both are needed to allow for maximum applicability to a population
• The precedent of assigning the level of acceptance/importance is highly dependant upon the question being answered
Moderator Comments

Key Areas of Controversy

• Causality
  – What level of certainty is necessary?
• Generalizability
  – Are the results fit for purpose?
  – How will treatment patterns affect outcomes?
• Patient-centeredness
  – How do the results inform patient choice?

Evidence Investment Options

• Clinical Trials
  – Phase 3 vs. Placebo
  – Phase 3b/4 vs. active comparator
  – Pragmatic trials
• Registries
  – Product vs. disease
  – Linked claims, PRO, clinical data
• Retrospective observational
  – EHR/registry mining
  – Claims
• Economic models
  – Trial-based
  – Mixed treatment comparisons

Discussion