

Assessment in RCT's of Behavioral Interventions

Linking Hypotheses, Outcomes and Assessment Measures

Nina R. Schooler, Ph.D.

Principle I

- Study hypotheses should be framed so that they translate to measurable variables
 - Example:
 - Pts who receive Cognitive Adaptation Training will report fewer psychotic, depressive and negative symptoms, higher levels of adaptive functioning and lower rates of relapse

Principle II

- Variables should be operationally defined by assessment measures
 - Example:
 - psychotic, depressive and negative symptoms
 - Brief Psychiatric Rating Scale
 - adaptive functioning
 - Multnomah Community Adjustment Scale

Principle III

- Assessments should be defined in advance and linked to domains of outcome in the study protocol and operations manual
 - example
 - psychosis BPRS items
 - suspiciousness
 - conceptual disorganization
 - unusual thought content

Principle IV

- Timing of assessments should capture the time course of anticipated change
 - Example - CARS clinical trial
 - psychosis symptoms measured weekly for first five weeks, then monthly
 - cognitive functions measured at baseline and six months

Principle V

- Assessment instruments should be “attractive” to reviewers and assessors
 - Established , with a history of use in the field
 - Document reliability
 - Published inter-rater
 - Study inter-rater
 - Document validity
 - Face
 - Discriminant

Universal Assessment Measures

- Definition of patient population
- Information regarding “Refusers”
- Randomization
- “Housekeeping”
- Adverse Events/Intercurrent Medical Illness
- Quality of Life

Definition of Patient Population

- Inclusion/exclusion criteria
- Diagnosis
- Other defining characteristics
- Refusers and generalization

Characterizing the Trial Population

- Outcome modifiers
 - Demographic information - age, gender, race, marital status, social class
 - Illness course - duration of illness, hospitalizations, prior treatments, treatment response
 - Personal factors - expectations of treatment, attitudes toward medication/other treatments, prior adherence

Refusal Reasons

- Example options
 - Experimental research study
 - randomized treatment
 - denial of need for treatment
 - denial of illness
 - study length
 - desire for treatment in another setting
 - uncooperativeness

Mechanics of Randomization: I

- Threats to Randomization
 - Assignment is known before individual is randomized
 - Assignment is not well concealed - the envelope please
 - In a non-blind study, participant withdraws once assignment is known
 - Randomization code can be broken by an interested party

Mechanics of Randomization: II

- Avoiding the threats:
 - Always
 - **Randomization lists prepared in advance**
 - **Record subject number on randomization form**
 - Multi-center studies
 - Centralized randomization Unit
 - Single Site Studies
 - **The envelope please**

“Housekeeping” Forms: I

- Visit form checklist
 - study time point
 - date
 - completed
 - yes
 - no, why not
 - procedures or other forms completed

“Housekeeping” Forms: II

- Medication Log - Strategies
 - Record medication changes
 - Start, dose change, discontinuation
 - Record dosage at fixed time points
 - Match assessment points

“Termination” Form

- Date of Last Visit
- Date of Study Termination
- Reason for Termination
 - Completed study
 - Worsening of clinical condition
 - Withdrawal of consent
 - Medical reason unrelated to target condition
 - Became ineligible
 - Other

Quality of Life Assessment

- General instruments to provide comparability to other RCT's
 - SF 36
- Specific instruments to address quality of life issues for the population/question
 - physical mobility in spinal cord injury patients
 - school setting in children

Adverse Events

- Every RCT should record adverse events
- General recording instruments readily available
- Specific events that may be related to population may also be indicated

Summary

- Design and choice of assessment instruments defines and RCT
- The assessment strategy brings the hypotheses and design to life
- The details of operation insure the integrity of a trial
- Multi-center trials magnify these needs