# The Use of Quantitative Methods in Practice-Based Research

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# Workshop on Practice-Based Research:

## My Main Objectives:

A. What is Practice-Based Research?

B. What are the main Goals of Research?

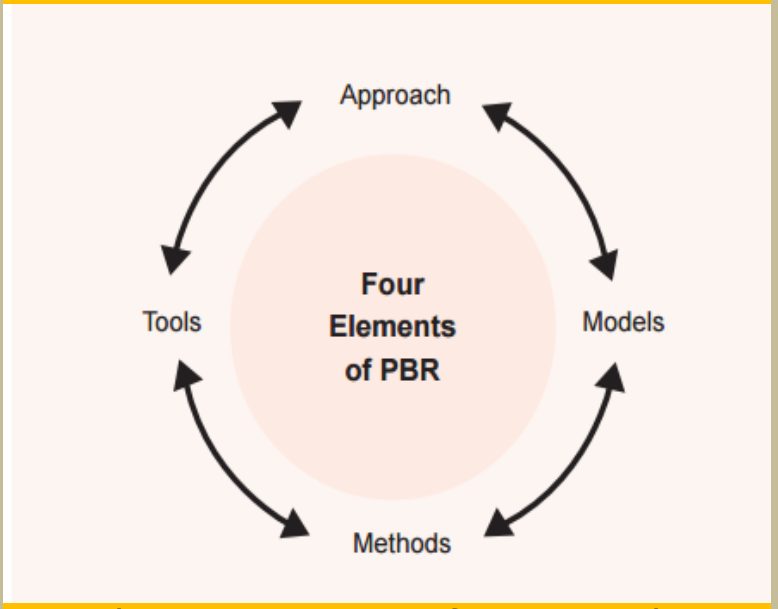
C. How to Select an Appropriate Research Design?

D. What to ask and bring to your Statistical Consultation Meeting?

# A: What is Practice-Based Research?

- Research is the empirical investigation of the relationship between or among several variables.
- Practice-based Research is an original investigation undertaken in order to gain new knowledge partly by means of Practice and the outcomes of that Practice.

**Four Elements of Research within any PBR:**




(Rowitz, L and Telfair, J 2005)

# A: What is Practice-Based Research?

- Approach: there are multiple methodologies that may be qualitative and quantitative.
- Model: is the structured format and design that systematizes and operationalizes the research approach.
- Methods: are the structural guidelines used to implement the research model.
- Tools: Tools are the instruments or means used within a given research method.

# Statistics, good, bad and ugly:

**Good:** Statistics can force us to look at data and facts rather than relying on opinions.

1. Statistics are the bridge between raw data and knowledge and understanding.

**Bad:** Statistics are confusing.

1. Statistics are even more confusing without graphics.
2. No amount of statistical analysis can ever produce certainty.

**Ugly:** “There are three kinds of lies: lies, damned lies, and statistics.” (Benjamin Disraeli )

1. Statistics are often misused to dismiss opposition.
2. People have a tendency to overlook or ignore statistics that contradict their own beliefs.

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# B: Main Research Goals:

1. To collect data that are free of bias, especially treatment-related bias.
2. To draw valid conclusions concerning the effects of an independent variable.
3. To make valid generalizations to populations and setting of interest.

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# C: How to Select an Appropriate Research Design?

1) What kind of research is being done?

2) What kind of questions will be tested?

3) What kind of data are required?

4) What kind of sample and how many subjects (power) will be needed?

## C: How to Select an Appropriate Research Design?

### 1: What kind of research is being done?

1. Data Type: Qualitative, Quantitative or Mixed method (Qualitative and Quantitative together).
2. **Main Objective:**
    i. **Feasibility,**
    ii. **Efficacy,**
    iii. **Effectiveness**.
3. Intervention Type: Observational & Experimental.

- Most of these divisions are not mutually exclusive.

# C: How to Select an Appropriate Research Design?

## I. Feasibility study.

- These are Pilot studies that are used to ensure that the theories or methods behind the research idea are sound, as well as to “work out the kinks”.
- They should not be used to test hypothesis.
- They should not be used to estimate sample size or power computations
- The standard error of the estimate of the effect size in these studies are so large, that the study will be aborted or will be underpowered.

# C: How to Select an Appropriate Research Design?

## II. Efficacy study.

- Are focused on the ability of a treatment to achieve the desired effect.
- Are focused on the degree to which an intervention accomplishes the projected outcomes.
- Popular method and trusted by researchers.
- Are highly controlled and methodologically developed (RCT).
- Are time-consuming and expensive.

# C: How to Select an Appropriate Research Design?

## III. Effectiveness study.

- They look at how much benefit "actual" subjects gain from "real-life" therapy.
- Subjects who have already begun (and possibly completed) treatment are surveyed.
- They are asked detailed questions about their treatment and its effectiveness.
- They reflect the full spectrum of disease, comorbidities, variable compliance rates, and use of other medications
- Most effectiveness studies are essentially surveys.
- They are much less time-consuming and less expensive to perform.

## C: How to Select an Appropriate Research Design?

### Efficacy Studies

1. Done usually in research facilities or in tertiary care settings.
2. Surrogate outcomes (i.e. scores, laboratory data) are frequently used.
3. Study duration is often limited.
4. Sample size are usually based on effect size.
5. They usually exclude protocol violators.

### Effectiveness Studies

1. Settings usually reflect the initial care facilities available to a diverse population with the condition of interest.
2. Primary outcome should capture the net effect on a health outcomes, using objective scales to measure their impact on health.
3. Study duration is often long to reflect the clinical setting.
4. Large sample size to detect at least a minimally important difference on a health-related QOL scale.
5. They are always done on an intent-to-treat basis.

# C: How to Select an Appropriate Research Design?

## 1: What kind of research is being done?

1. Data Type: Qualitative, Quantitative or Mixed method (Qualitative and Quantitative together).
2. Main Objective: Feasibility, Efficacy, Effectiveness.
3. **Intervention Type:**

   **I. Observational**

   **II. Experimental**

- Most of these divisions are not mutually exclusive.

# 1: What kind of research is being done?

## Intervention Type:

- **Experimental**
  - Controlled
    - 1: Self-Controls (Waiting List ; Before/After)
    - 2: Independent Concurrent Controls
    - 3: Crossover Studies
    - 4: With External Controls
  - Uncontrolled
- **Observational**
  - 1: Case Series
  - 2: Case-Control (Retrospective)
  - 3: Cross-Sectional (Prevalence)
  - 4: Cohort (Prospective)

# OBSERVATIONAL STUDIES:
## 2: Case-Control (Retrospective Studies)

Determining Differences in:

- Life Style
- Diet
- Activity level
- Medication
- Family History
- Genetics

PRESENT:

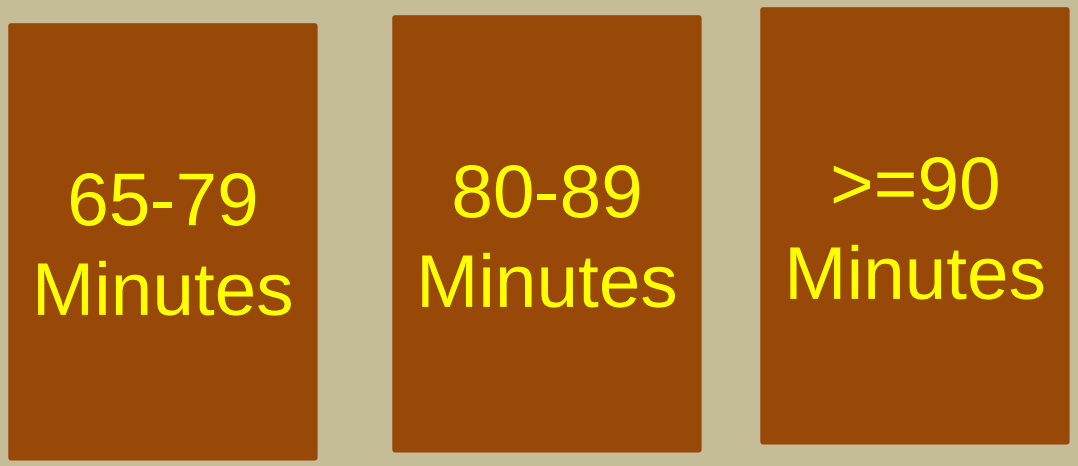
Absence or Presence of an outcome

PAST

Time

# OBSERVATIONAL STUDIES:
## 3: Cross-Sectional (Prevalence Studies)

Question: "what is happening?" right now.

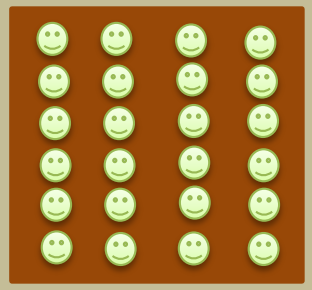



Average minutes per day spent in light activities

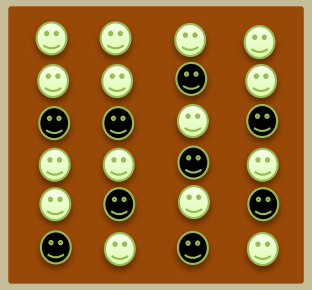
# OBSERVATIONAL STUDIES:
## 4: Cohort (Prospective)

Question "what will happen?"

Group of Interest: i.e. Low Activity

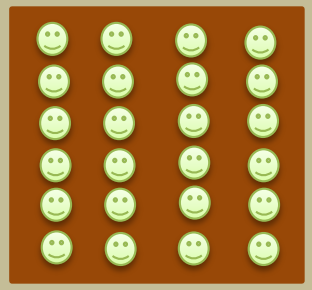


Followed over time



Compare Outcomes

Comparison Groups: i.e. High Activity



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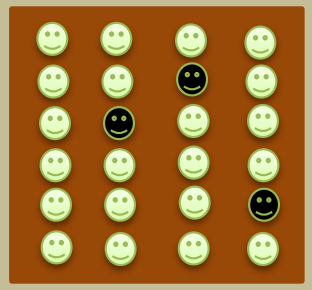
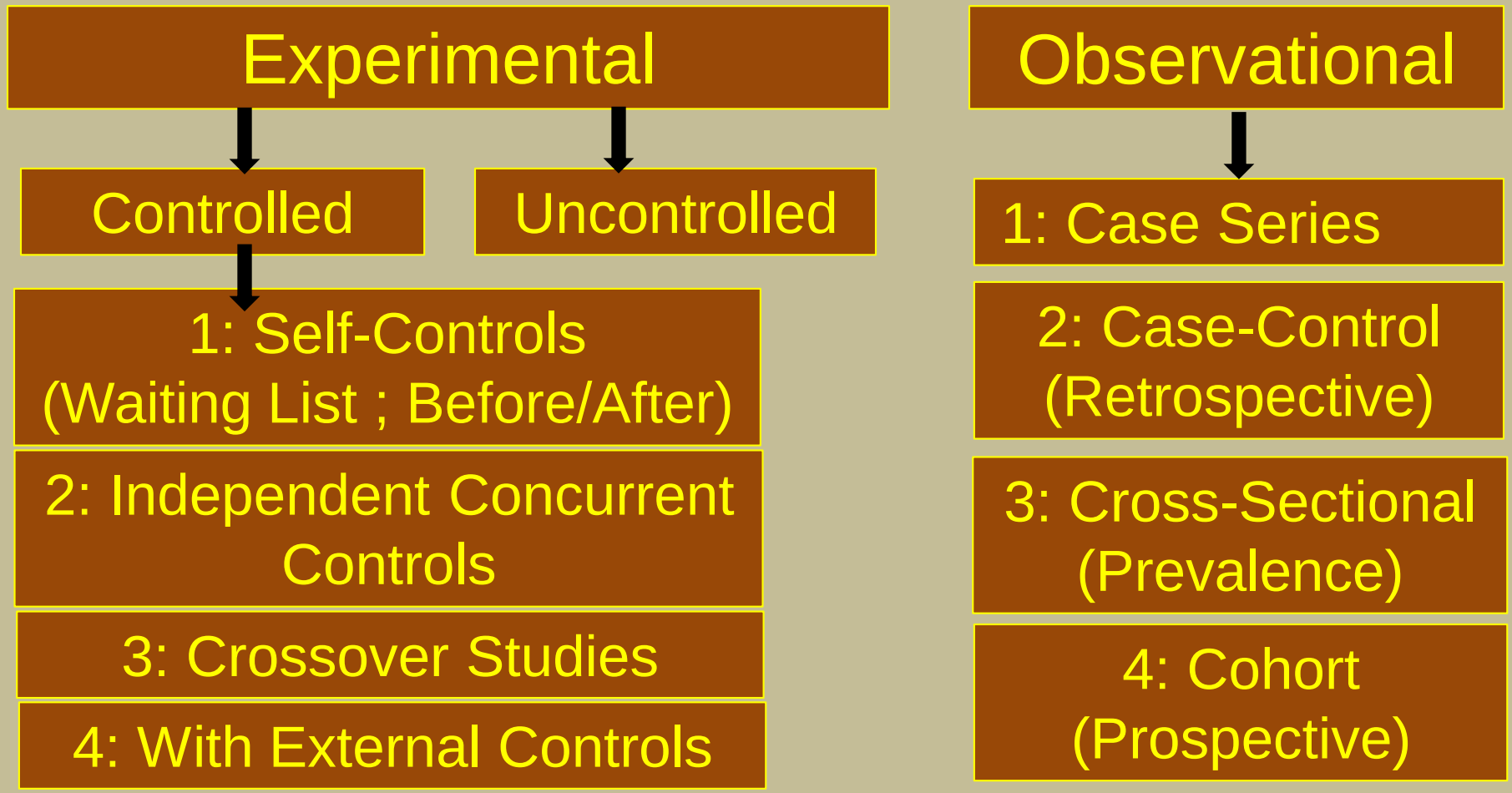


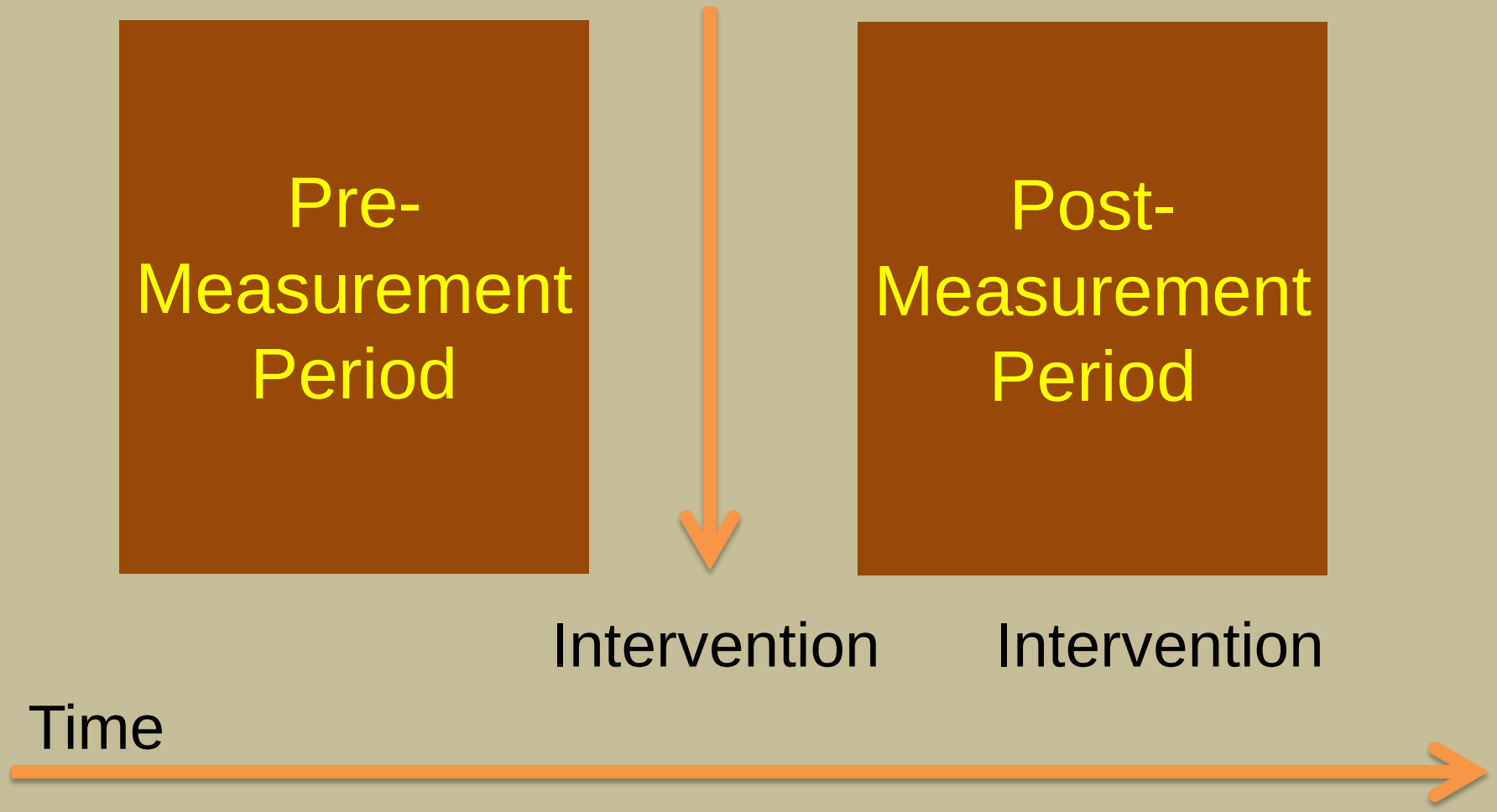
# 1: What kind of research is being done?

## Intervention Type:



# CONTROLLED INTERVENTIONAL STUDIES:

## 1: Studies with Self-Controls (Waiting List ; Before/After)



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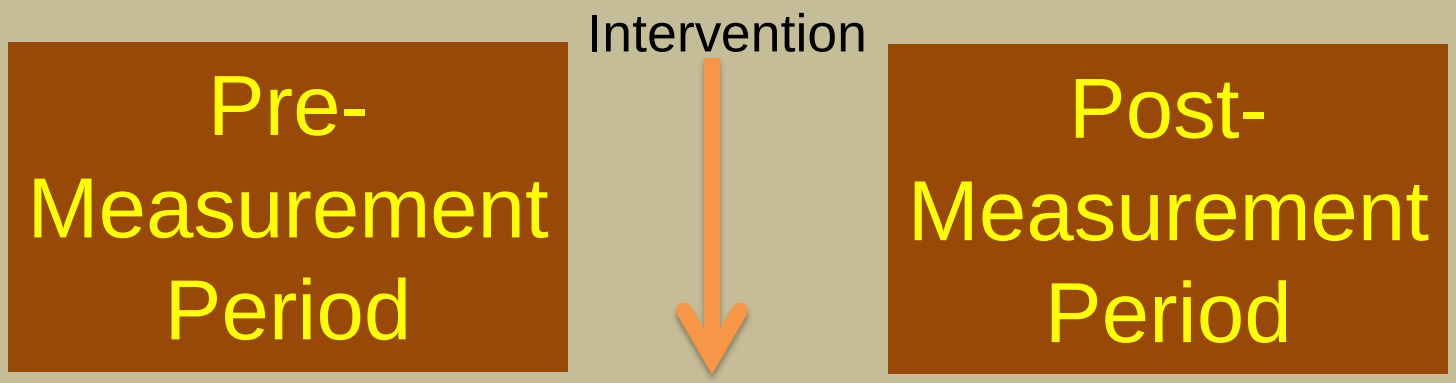
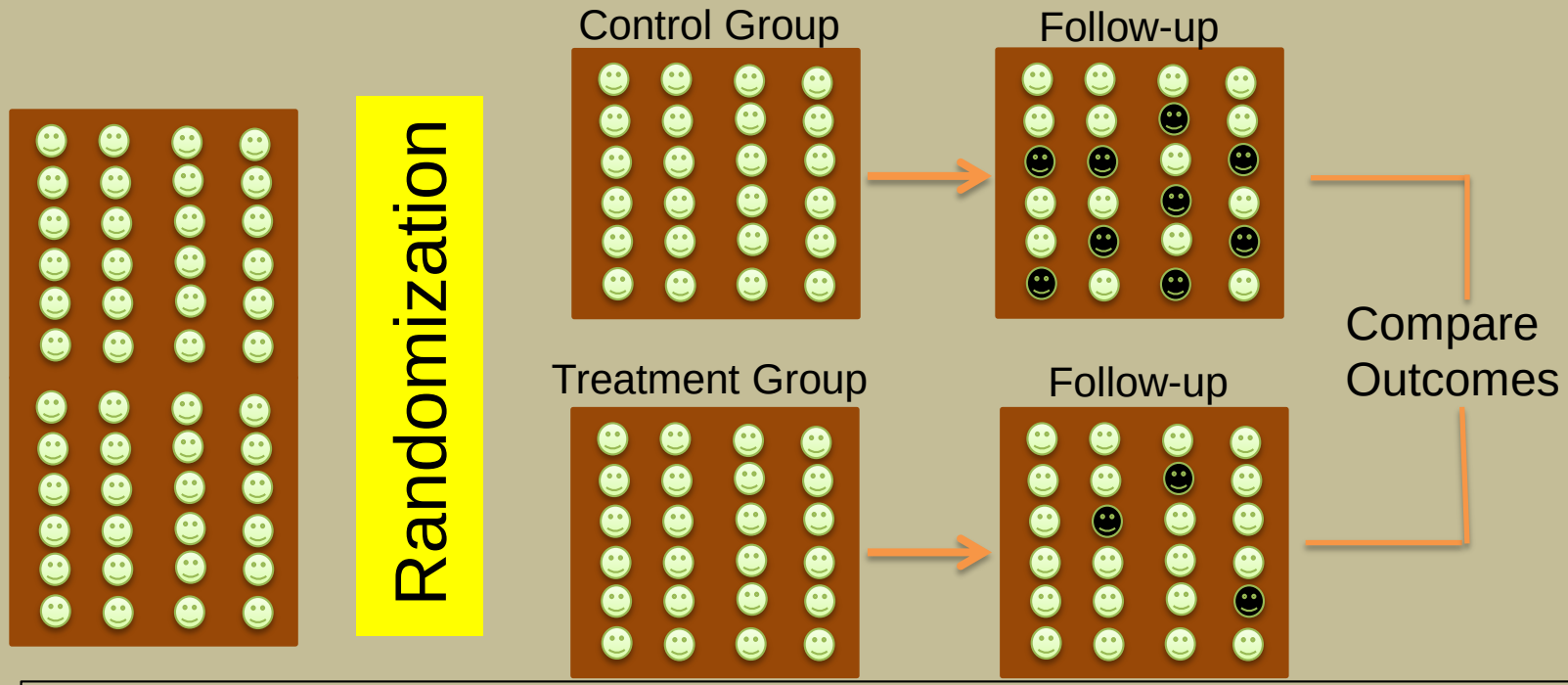



Analysis Method:

| | |
|---|---|
| Continuous Outcomes: | Paired t-test |
| | Repeated Measures ANOVA |
| Frequency Outcomes: | Repeated Measures Chi-Square ($x^2$) |
| | McNemar's $x^2$ |
| Ordered Outcomes: | Sign Test |
| | Friedman Test |

# CONTROLLED INTERVENTIONAL STUDIES:

## 2: Studies with Independent Concurrent Controls




### Analysis Method:

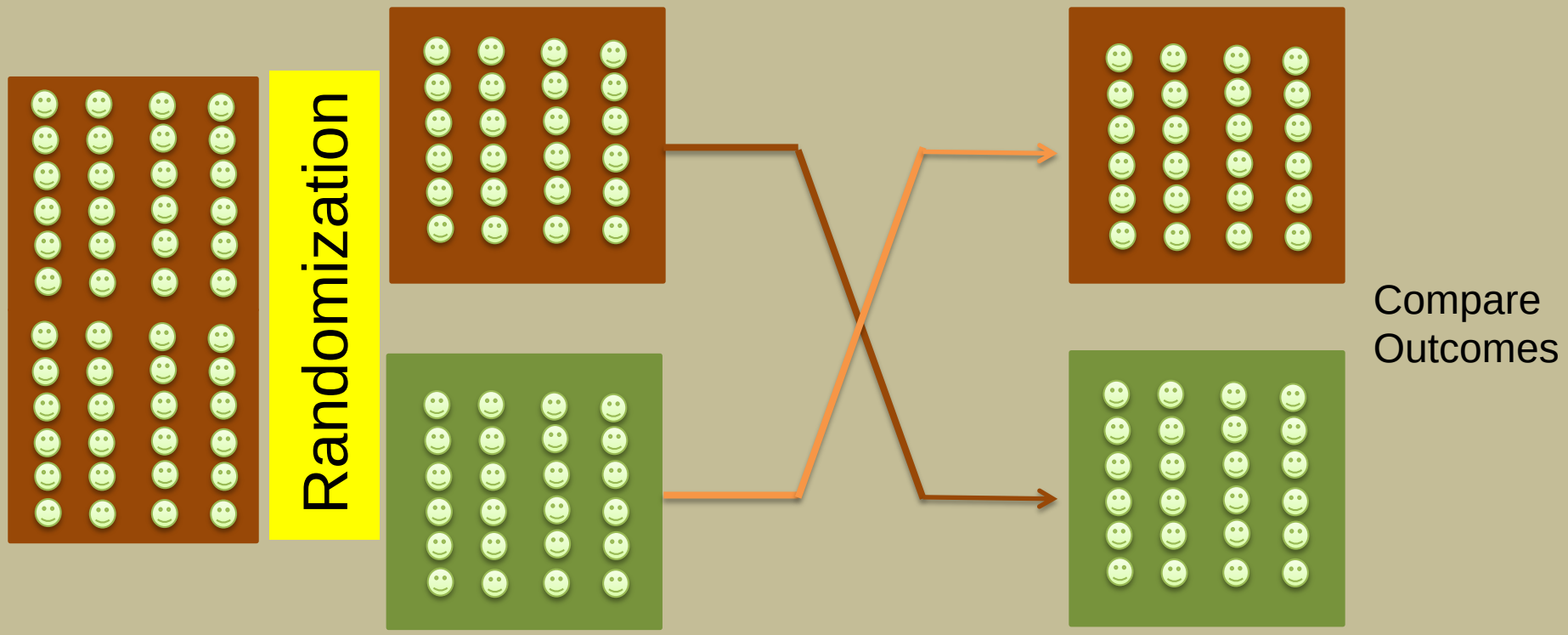
Continuous Outcomes: t-test or ANOVA

Frequency Outcomes: Chi-Square ($x^2$)

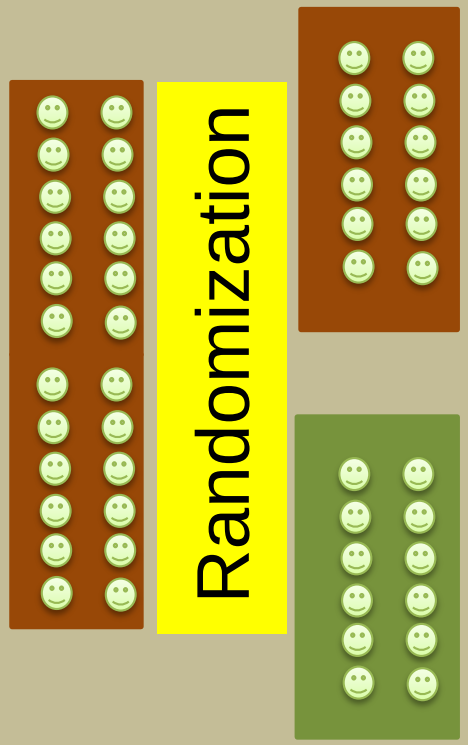
Ordered Outcomes: Rank-Sums and Kruskal-Wallis tests

# CONTROLLED INTERVENTIONAL STUDIES:

## 3: Cross-Over Studies

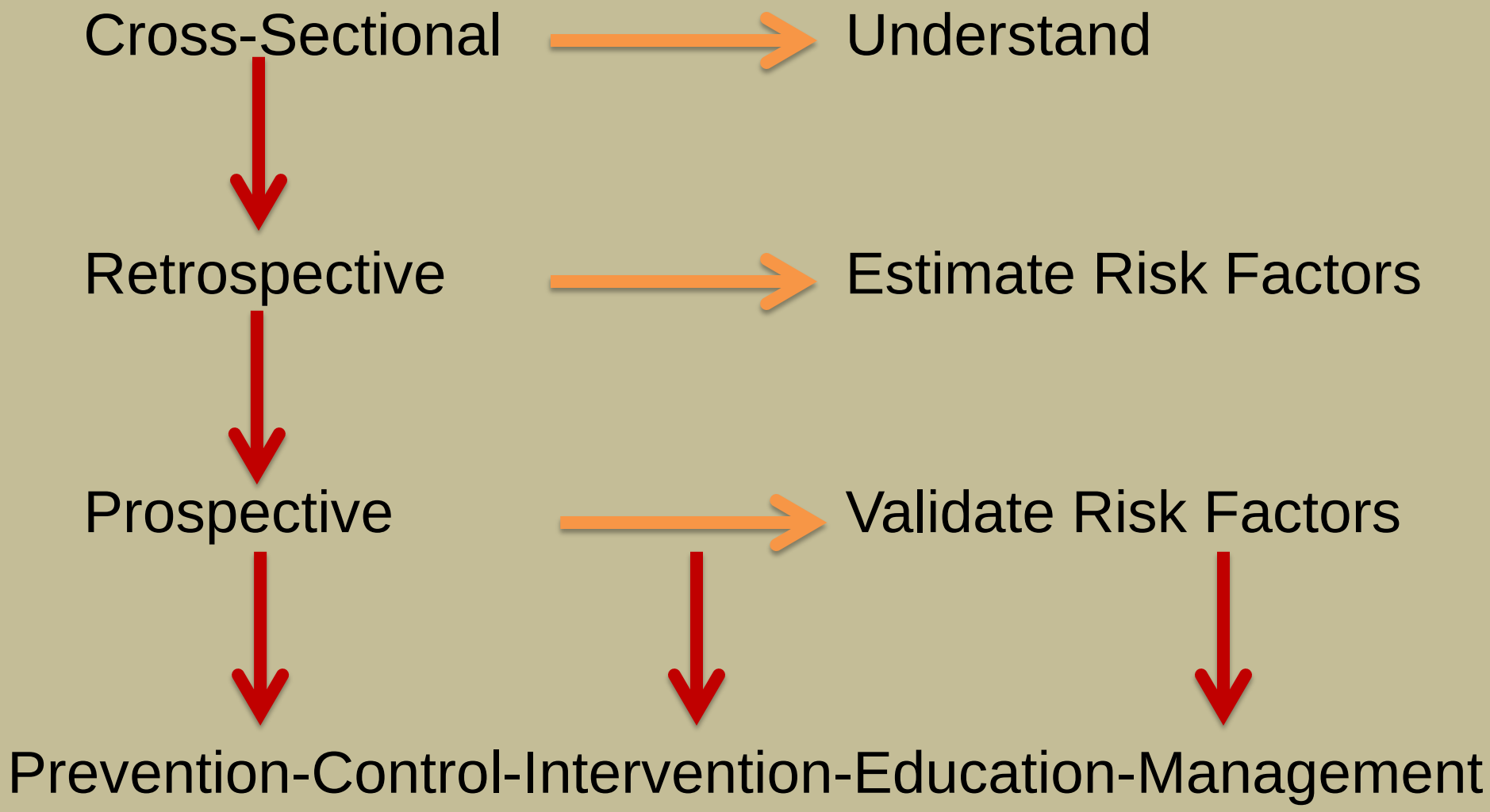


# CONTROLLED INTERVENTIONAL STUDIES: Randomization




1. Completely Randomized (CR).
2. Stratification is the process of grouping subjects into relatively homogeneous subgroups before sampling and randomization.
3. Urn randomization is an adaptive allocation strategy that adjusts the probability of assignment to condition to achieve optimal multivariate equivalence of treatment groups across several covariates.
4. Equipoise-Stratified randomization the clinician and patient in principle define the list of specific study treatments that are acceptable and of rough parity. This list is called the “equipoise stratum”.

# 1: What kind of research is being done?



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# C: How to Select an Appropriate Research Design?

## 2: What kind of Questions will be tested?

**A. Estimation**

**B. Hypothesis Testing**

1. Is it about association among variables, prediction or groups differences?
2. For group differences: A Single Group or Two or more groups? If two or more:
   - Independent Groups
   - Dependent Groups
3. What are the costs for false positive or false negative results?
4. Is it directional or non-directional (two-tailed)?

# C: How to Select an Appropriate Research Design?

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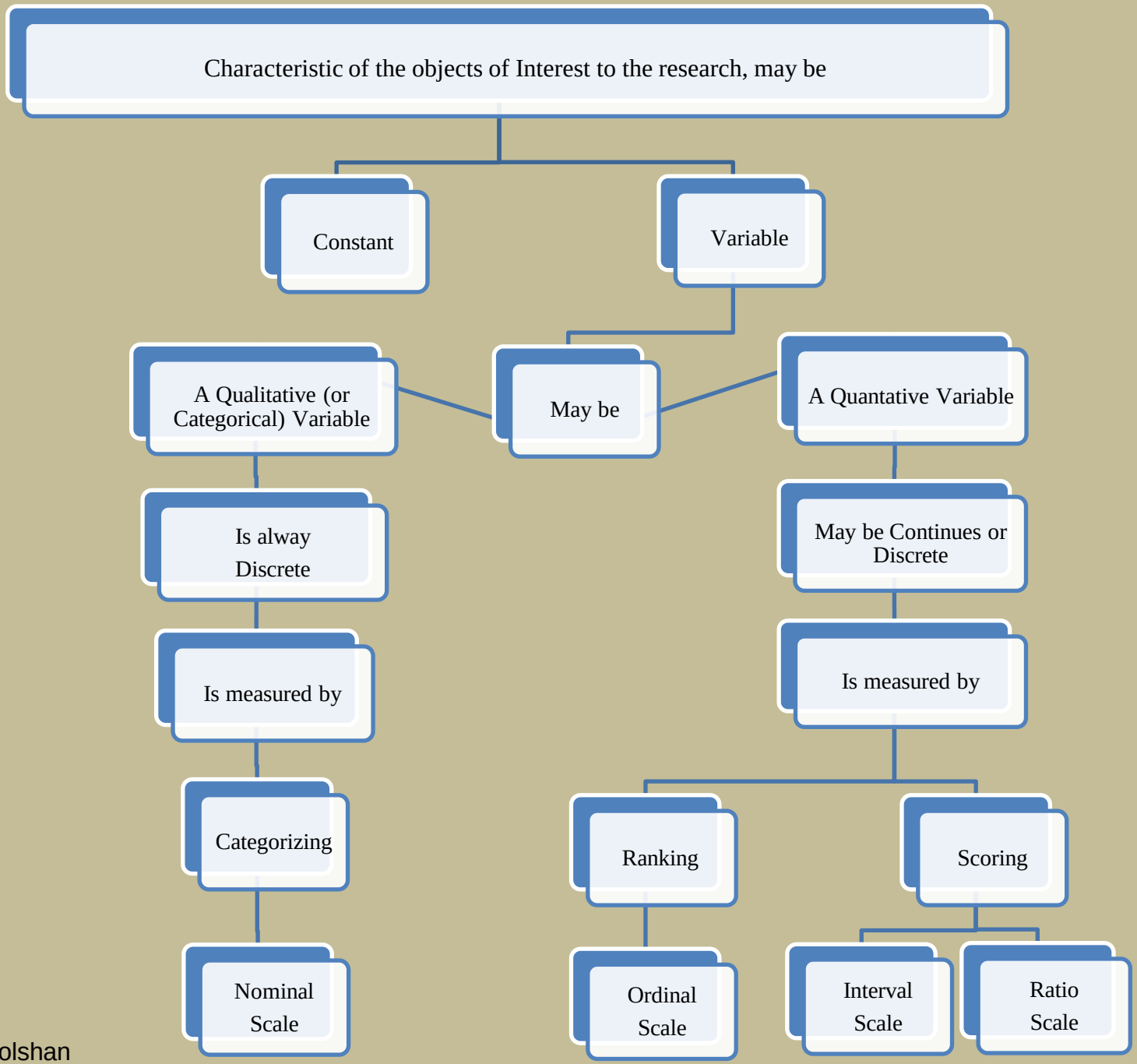
Characteristic of the objects of Interest to the research, may be
Constant
Variable
May be
A Qualitative (or Categorical) Variable
A Quantative Variable
Is alway Discrete
May be Continues or Discrete
Is measured by
Is measured by
Categorizing
Ranking
Scoring
Nominal Scale
Ordinal Scale
Interval Scale
Ratio Scale

# C: How to Select an Appropriate Research Design?

1) What kind of research is being done?

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# C: How to Select an Appropriate Research Design?

## 4: What kind of sample and how many sample (power) will be needed?

1. Should subjects be observed under more than one treatment level?
2. How many times should one subject be measured?
3. Should subjects be stratified into homogeneous blocks?
4. What is the practical treatment effect?
5. What statistical effect size does the treatment effect convert to?

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# D. What to ask and bring to your Statistical Consultation Meeting?

## I. Most common questions:

1. Number of subjects:
   - How many subjects do I need?
   - Is XX enough subjects?
2. How do I do …?
3. Can you do these analyses?
4. Does it sound okay/make sense? (NO)
5. Can this be fixed? (NO)
6. Which result is correct?
7. How do I read this output?

# D. What to ask and bring to your Statistical Consultation Meeting?

## II. Better questions:

1. What is the effect size for this clinical change?
2. Is/was the number of subjects sufficient for the expected effect size?
3. Is/was the design appropriate for the Hypothesis?
4. Is the data type correct?
5. Are/were analyses correct based on design and hypotheses?
6. Is/was the power sufficient?

# D. What to ask and bring to your Statistical Consultation Meeting?

## III. What to provide in your meeting with a Statistician:

1. Written description of the study.
2. Research Questions and Hypotheses.
3. Study Design:
    - Number of groups
    - Group definitions
    - Number of time points, their correlations
    - Number of variables
    - Variables types, description and scoring method
    - Sites/Randomization/Stratification methods

# D. What to ask and bring to your Statistical Consultation Meeting?

## III. What to provide in your meeting with a Statistician:

4. Relationship between variables & hypotheses.
5. Relationship between analyses & hypotheses.
6. Effect size documentation (Clinical/Statistical).
7. If study has been completed:
   - How was the study conducted and its duration.
   - Number of subjects screened, rejected, dropped and finished.
   - How data was managed (quality control).

# D. What to ask and bring to your Statistical Consultation Meeting?

**Consult before any data collection!**

# Workshop on Practice-Based Research:

1. Remember Your Research Goals.
2. Select an Appropriate Design:
    i. Know your Research Type
    ii. Know your Hypotheses/Questions
    iii. Know your Data Type
    iv. Know your Sample (Type, required size)
3. Consult before any data collection!

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