Important Factors in Designing Clinical Trials

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To Do List for Research Project

- Define your research question
- Assess adequacy of current information
- Choose appropriate clinical design
- Select outcomes
- Consider new technologies to conduct trials
- Avoid common mistakes
- Getting value for money

Defining Research Question

- Be specific about what you want to know
- Challenging to test dietary supplement claims
- Efficacy vs. effectiveness
- Evaluating harm- more difficult
- Exception to this rule: obtaining safety data

Do you know enough to proceed?

- Why review the literature?
- Characterization of your test product
- Adequate safety data
  - Traditional knowledge
  - Animal data & pre-clinical
  - Human data
- Bioavailability & Pharmacology
  - Fasting vs. non-fasting
  - Formulation
  - Dosing schedule

Do you know enough to proceed?

- Dose ranging study
  - Range from 1/2 expected dose to double expected dose
- Outcome tools
  - What has been used before?
  - Do validated methods exist?
  - Can we use these same methods?
- Feasibility of design?
  - Can this project actually be run and completed
- Can you ever skip all this?

RCT: Is that all there is ?

- When is an RCT NOT appropriate?
- Value of a chain of evidence (CS)
- What constitutes the appropriate control group for a clinical trial?
- Active vs placebo control
- How many patients to enroll
- Consideration of different trial designs: case series, cohort, n of 1, etc.

Choosing Outcomes Wisely

- Answer the research question
- Choose evaluation interval wisely
- Use patient centered outcomes
- Use validated instruments where possible
- Primary vs secondary endpoints: pro's & con's
- Blind assessors to the group assignment for controlled trials
- Opportunity to collect safety data
- Less is more: only collect what you need

Avoiding Common Mistakes

- Not enough preliminary data to conduct the trial
- Not performing a reasonable effect size calculation (enroll too few or too many subjects)
- Too many outcomes
- Not using an appropriate outcome
- Failing to consider dropouts in study design
- Performing the wrong analysis on the data

New Technologies

- Using the web to your advantage
  - Advertising trials
  - Preliminary screening
  - Collecting patient based data
  - Conducting complete trial
- Characterization of test materials
- Changes in design

Summary & Questions