Clinical Trials: a Patient Perspective

Ron Ternoway
IWMF Ed Forum
Phoenix, AZ
May 20, 2017
It's NOT about YOU!

WE WANT YOU!
The Scientific Method

The Scientific Method as an Ongoing Process

- Make Observations
  - What do I see in nature? This can be from one’s own experiences, thoughts, or reading.

- Think of Interesting Questions
  - Why does that pattern occur?

- Develop General Theories
  - General theories must be consistent with most or all available data and with other current theories.

- Develop Testable Predictions
  - If my hypothesis is correct, then I expect a, b, c, ...

- Gather Data to Test Predictions
  - Relevant data can come from the literature, new observations, or formal experiments. Thorough testing requires replication to verify results.

- Refine, Alter, Expand, or Reject Hypotheses

- Formulate Hypotheses
  - What are the general causes of the phenomenon I am wondering about?
What is a Clinical Trial?

Follow these trial directions exactly... Take a blue pill Monday and Wednesday at 10 a.m. with 6 ounces of yogurt. Take 2 red pills on Tuesday and Thursday at 6 p.m. and don’t eat for 3 hours. Take a green pill Friday and Saturday at midnight with no food until 6 a.m. On Sunday take one of each color at noon with bacon and eggs. Got it?

Following exact directions is vital!

I’ll just take ’em all Monday morning and not worry about the rest of the week.

Careful controls are the backbone of clinical trial success.

a scientifically controlled study of the safety and effectiveness of a therapeutic agent using consenting human subjects
The 3 phases of clinical trials

- Phase 1 – establishes safe dosage of drug
- Phase 2 – determines efficacy of new drug
- Phase 3 – compares new drug against current therapies
Possible Phase 3 Trial Outcomes

- **Positive** trial -- new treatment was at least as effective as expected.
- **Non-inferior** trial -- new treatment was not significantly less effective (and might be better) than standard treatment.
- **Inconclusive** trial -- new treatment is neither clearly superior nor clearly inferior to standard treatment.
- **Negative** trial -- new treatment was not as effective as expected.
So what option is best for me?

The decision to participate in a clinical trial should be a thoughtful process in which patients carefully evaluate many aspects of the clinical trial option.
Personal Considerations

Eligibility for Clinical Trial

What types of eligibility issues could be barriers for me entering a specific clinical trial?
Personal Considerations

Protection of Patient Rights

How are my rights protected during a clinical trial?
Personal Considerations

Pros and Cons of Clinical Trial vs Standard Care

How do I make the right treatment decision?
Personal Considerations

Requirement for Travel

Am I willing and able to travel for treatment and testing?
Personal Considerations

Requirement for Medical Tests

Am I willing to undergo extra testing that may be required, such as bone marrow biopsies, CT scans and frequent blood tests?
Personal Considerations

Insurance Coverage

If my health insurance will not pay for additional testing, does the trial sponsor cover the cost?

Does my health insurance pay for routine tests and drugs for side-effects caused by the trial regimen?
Personal Considerations

Risks of Trial Treatment

Am I able to cope with the known and unknown risks of the trial drugs?
Personal Considerations

Continuation of Treatment
Beyond Clinical Trial

If the drug is working for me, can I continue treatment after the end of the trial?
Personal Considerations

Why am I doing this trial?

- Desperation – out of options
- Altruism – advance research for others
- Blind Optimism – doc says this drug will cure me!
- Considered Optimism – my research says go for it
Before you decide...

What should I ask my doctor?

- What is the scientific rationale for using this therapy for WM?
- What is the trial phase, and what are the goals or endpoints?
- Has this drug been used on humans before? If so, side-effects?
- How is the treatment administered, and for how long?
- What is number & frequency of medical tests and clinic visits?
- How many extra tests are specifically for trial purposes?
- What's the likelihood that this therapy will help me?
- Are there approved or standard therapies we should try first?
Clinical Trials: a Patient Perspective

Questions?

Cartoons by Larry Axman (RIP)

(c) Tom Beer and Larry Axmaker 2012
http://www.cancer-clinical-trials.com

Of course I’d do anything to manage my cancer...chemo, radiation, rat hormones, or green cheese, but NO clinical trials for me!
Requirement for Travel

Out-of-pocket travel expense is typically not covered by the trial sponsor. In the US, the following organizations may help:

**Angel Flight** is a non-profit organization of pilots, volunteers, and friends who will arrange free air transportation for medical treatment. Volunteer pilots donate the use of their airplanes and operating expenses. For details: www.angelflight.com.

**Corporate Angel Network** is a non-profit organization that arranges free air travel for cancer patients to treatment centers by using empty seats on corporate business flights. For details: www.corpangelnetwork.org.

**American Cancer Society** provides Hope Lodges in over 35 locations in the US. These are free home-like housing options for cancer patients and caregivers. For information: www.cancer.org and type Hope Lodge in search box or call 1-800-227-2345. Free or reduced cost housing may be available through the local cancer facility.