

Clinical Trials: a Patient Perspective



Ron Ternoway
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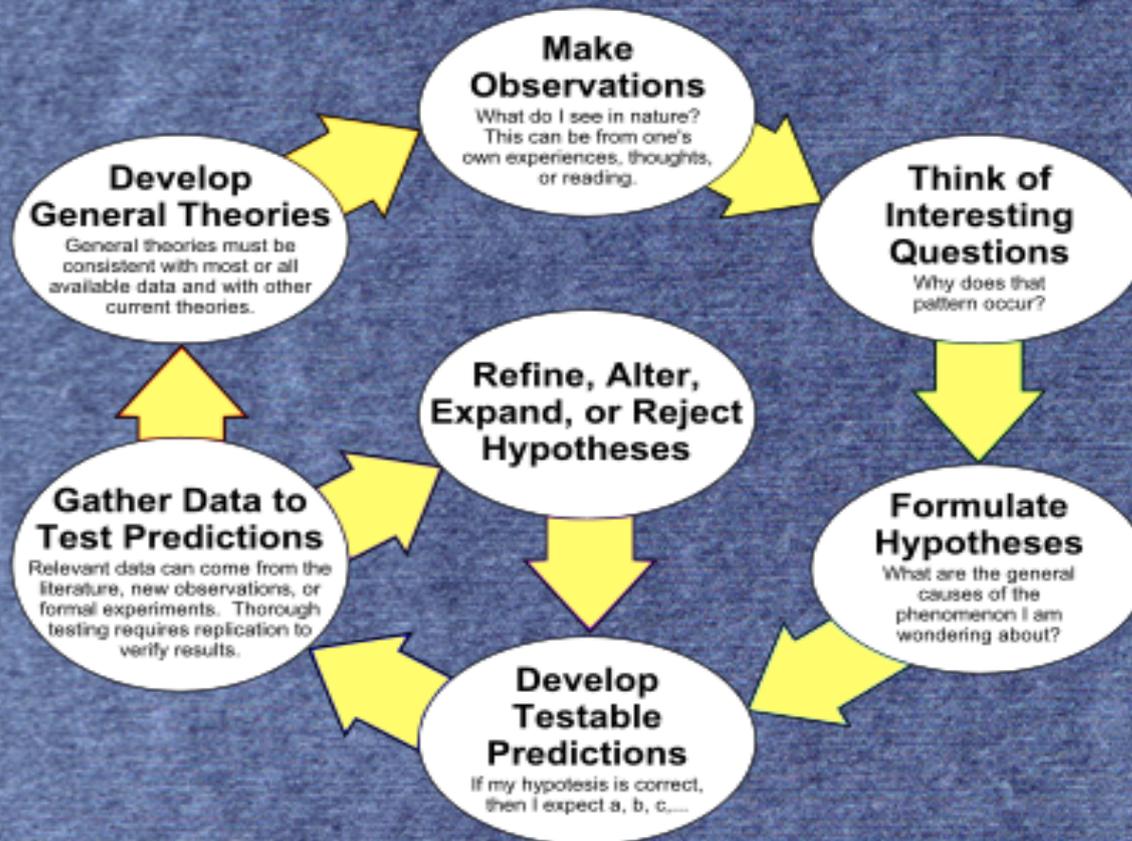
It's **NOT** about **YOU!**



WE WANT YOU!

The Scientific Method

The Scientific Method as an Ongoing Process



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!



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

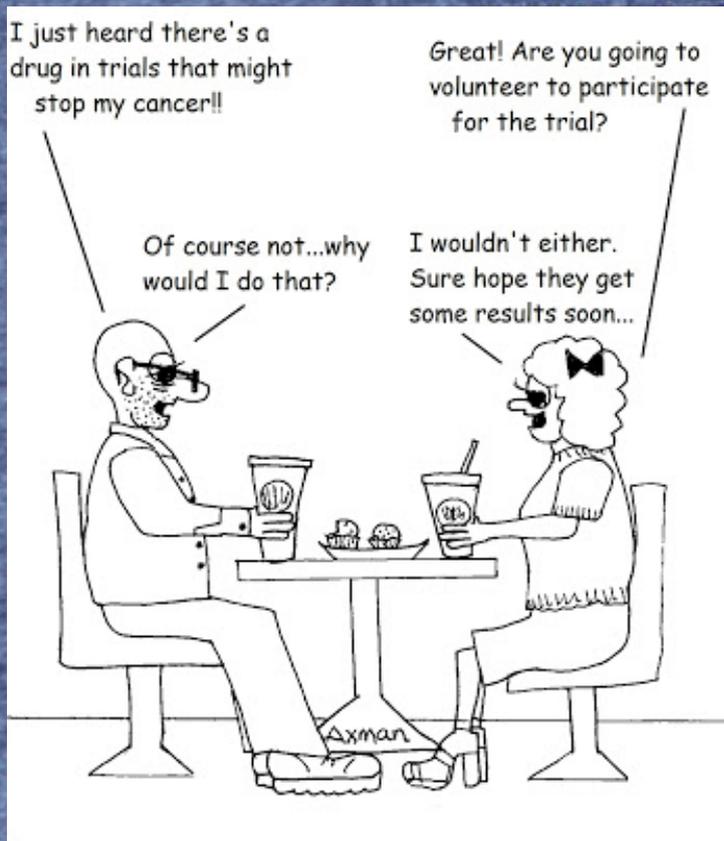
- Phase 1 – establishes safe dosage of drug
- Phase 2 – determines efficacy of new drug
- Phase 3 – compares new drug against current therapies



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.

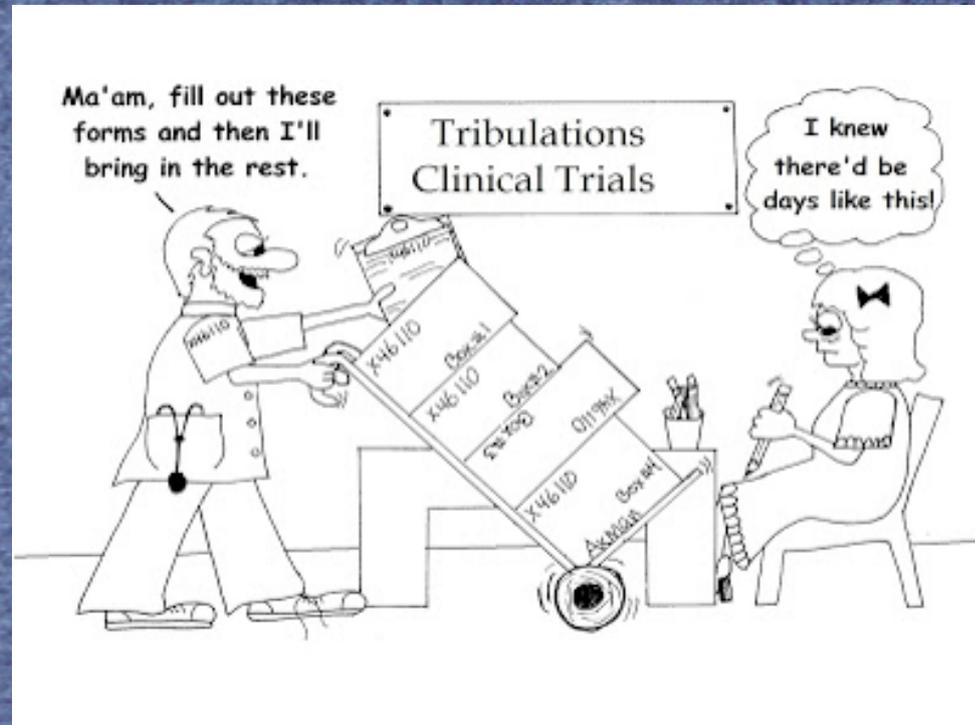
Personal Considerations



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

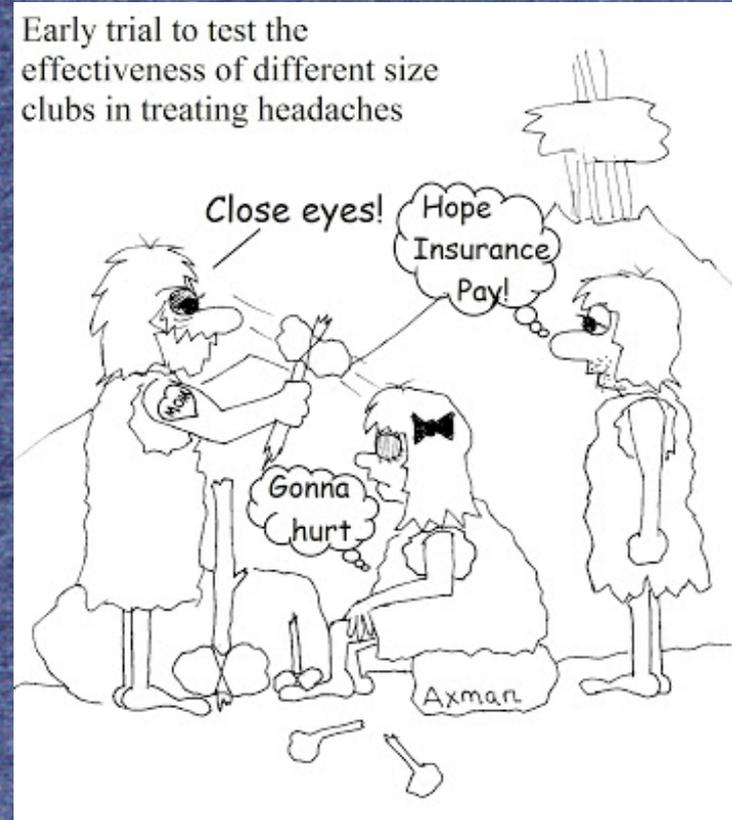


Requirement for Medical Tests

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

Personal Considerations

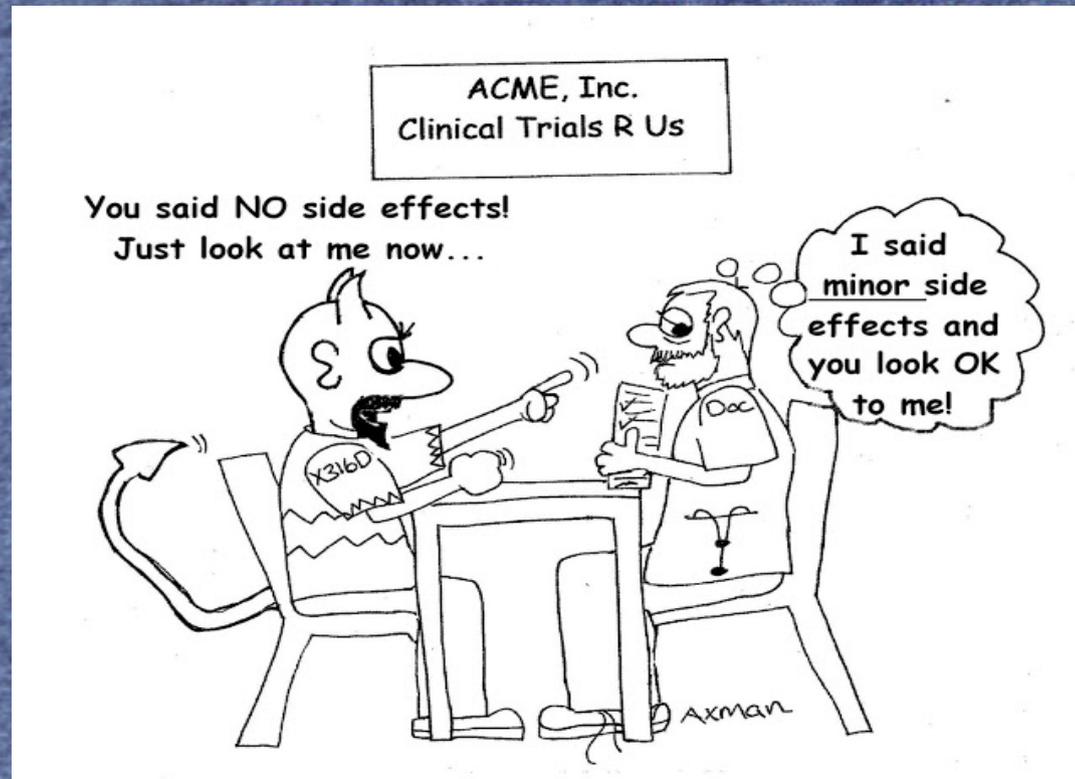
Insurance Coverage



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?

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



Cartoons by Larry Axman (RIP)

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

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 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**.