AN INTRODUCTION TO CLINICAL TRIALS

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Refresher: What is the goal of clinical trials?

A. Study the impacts, patterns, causes, and effects of health and disease
B. Examine how patients access health care services
C. Evaluate effects of a specific treatment or medication on the people’s health
D. All of the above
Phases of Clinical Trials

One
Small group of people (20-80)
Treatment and its safety studied for the first time

Two
Larger group of people (100-300)
Effectiveness and safety of treatment determined

Three
Large groups (1,000-3,000)
Safety information collected, and treatment compared with others

Four
Treatment approved and made available.
Researchers track risks, benefits, and safety.
Study Funding

- Studies are funded by sponsors

  - Sponsors can be people, institutions, companies, or other organizations that finance and manage the trial but often do not actually do the research
Important Terms in Clinical Trials: “Placebo”

**Placebo** - inactive product that resembles the treatment being tested. Used because researchers won’t know if a treatment works or if it is safe if there is nothing to compare it to.

- Not used if a lack of treatment can put you at risk!
- Used as a comparison to determine **effectiveness and safety** of the treatment.
- For example, infusion of belimumab drug versus an injection of saline (placebo)

- Helps answer two questions:
  1) Does the new treatment work?
  2) Does it do what it is expected to do?

- Often “placebo” arm receives the care their primary provider prescribes (not nothing)
Review: Example of Informed Consent Document

Informed Consent Document

• Study Details
  • What is being studied?
  • Who is paying for it?
  • IRB number
    • Shows that the study plan was reviewed by the IRB
  • Who is performing the study?
  • How can you contact them?
  • How many participants does the study need?
  • How many places will they try to recruit participants?

• Study Purpose
  • Why is this study being done?
  • Why is this study important?

- Begins by explaining the basics of the study
- “Who, What, Where, and Why”
Review: Example of informed consent document

- **Protocol (roadmap)**
  - What is the study design?
    - Will participants be “randomized” into groups?
    - Who will be “blinded”?
  - What will happen during the study?
    - Number of visits?
    - What will happen at each visit?
      - What information (medical history, examination, blood sample, questionnaires, treatment) will be collected or given at each visit?
        - The kind of information or treatment may be different at each visit
      - Are placebos used?

- The Informed Consent Document will then describe how the study will be done
• **Risks of the Study**
  - Common risks include:
    - Your health may or may not improve
    - Blood draw risk
    - Confidentiality risk
    - Pregnancy risk

• **Benefits of the Study**
  - Common benefits include:
    - Access to expert medical care
    - Learning more about a health condition you have

• Some risks and benefits are unknown

- The document will then provide new information that might impact your participation
  - **Risks** and **Benefits** of enrolling in the study
The document will explain the payment for the study and other treatments for the condition being studied:

- Researchers want to make sure that participants are choosing the best option for them.

The document will also explain what will happen if you become sick or hurt during the study:

- What will happen if you get hurt or sick from something in the study?
- What might be charged to your insurance?
- Only would happen if the event is due to a pre-existing condition or if you aren’t following the study procedure.
Review: Example of informed consent document

Informed Consent Document

- **Volunteer Participation and Withdrawal**
  - How can you tell the study staff you don’t want to participate in the study anymore?
  - Why might your doctor or the study sponsor want you to stop the study?

- **Confidentiality**
  - Permission to review your medical records
  - List of who may see the information collected during the study, including laboratory test results

- The document will then explain how you can **stop participating** in the study if you don’t want to continue

- Explains how your information will be kept **private**
Informed Consent Document

- **HIPAA Authorization**
  - Protections to respect privacy
  - From the “Health Insurance Portability and Accountability Act of 1996”
  - Lists who look at your medical record and study data
  - Tells you when the study team will stop collecting new data and when your consent for the study expires

- **Signatures**
  - Your consent
  - Person who reviewed and consented you

- Explains what the study team will do to protect your privacy
- Who will see the data and when they will stop collecting new data
- At the end, you will **sign** the document to consent
Let’s Revisit the Consent Form

- How do you think a description of a “placebo” treatment would show up in the study’s IRB and consent form?

- IRB- would have a detailed description of the placebo being used and its safety information

- Consent form- Research team would tell you if a placebo was being used in the study
**Blinding** - Study design meant to keep research team members and participants from influencing study results

**Single Blind**
Participants are not told which treatment they receive

**Double Blind**
Participant and Research Team don’t know which treatment each person received, and a person outside of the study keeps track

 Patients and their physicians can ALWAYS find out which treatment they are receiving if it is medically necessary or for safety reasons!
Important Terms in Clinical Trials: “Randomization”

- **Randomization**: Treatments are assigned to patients randomly (like a coin toss).

- Done to reduce the chance that scientists’ expectations of what should happen with a treatment will influence the results they see from the research (bias).
Treatments are Evaluated During the Trial

- *Effects and safety of treatments are compared during the trial.*
  - If one is better, trial is stopped so participants can get the new treatment.

- **SELENA trial- Tested if oral contraceptives were safe for lupus patients to take.**
  - At the occurrence of a lupus flare, the physician would assess whether or not it was safe for the study subject to continue

What has Caused Hesitation around Clinical Trials Participation?

- History of unethical treatment of certain populations
- Experiences of racism
- Concern of feeling like a “guinea pig”
- Strict exclusion criteria
- Fear of receiving the placebo arm
- Not much incentive
What has Caused Hesitation around Clinical Trials Participation?

- Fear of side effects from a new medication
- Lack of trust in the health care system
- Lack of time
- Hard to understand consent process
Which one is not a clinical trial?

A. A study looking at the risk of development of lupus associated with how close patients live to toxic waste sites

B. Testing whether or not a new drug works better than hydroxychloroquine to prevent lupus flares

C. Studying whether an in-person walking group is more effective at getting people to exercise than a mobile phone app

D. A study trying to determine whether fatigue among lupus patients is less among patients receiving vitamin D supplements compared to placebo
What Should You Learn Before Enrolling?

- **Risks**
  - Potential harms and risks, as well as the chance of harm or risk occurring
  - Treatments may have unpleasant or life-threatening effects
  - Study may require a larger time commitment than standard medical care
What Should You Learn Before Enrolling?

- **Potential Benefits**
  - Contribute to studies developing new treatments/procedures
  - Chance to help others in the future and to advance science
  - Access to new treatments before they are available to the public
  - Access to regular medical attention from a full research team
What Questions Should You Ask about Study Safety?

- What is the purpose of the study?
- Why do the researchers think that their approach will be effective?
- Who reviewed and approved the study?
- Is patient safety monitored throughout the trial?
- What happens if in the middle of the study, one group is doing much better (or worse) than the other?
- What are the short and long term risks and benefits?
What Questions Should You Ask about Coordinating Trials and Normal Healthcare?

- Will you be able to continue your normal medications and treatment during the trial?
- Who will be in charge of your care, and where will the care occur?
- Will your doctor know exactly what you are receiving and the results of any tests that are done?
What Questions Should You Ask about the Study’s Costs?

- How will the study affect your daily life?
- Will you have to pay for any portion of the trial, and how much will it cost?
- Is your parking or transportation reimbursed?
- How many extra visits will you have to make?
- Who can help you answer questions from your insurance company or health plan about the trial?
What Questions Should You Ask about Confidentiality and Contacts?

- Who has access to your information?
- How will this information be protected?
- Will it be shared with anyone?
- Who can you contact with any questions, concerns, side effects?
Questions? Thank you!
Words to Remember

- **Randomization**: treatments are assigned to patients randomly (like a coin toss)
- **Bias**: scientists’ expectations of what should happen with a treatment influence the results they see
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