National Institute of Neurological Disorders and Stroke

Clinical Trials 101

Adam L. Hartman, MD, FAAP, FAAN, FANA, FAES

Program Director, Division of Clinical Research, NINDS

DEE-P Webinar

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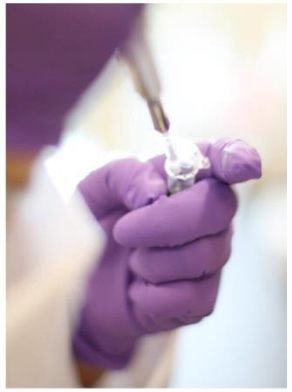
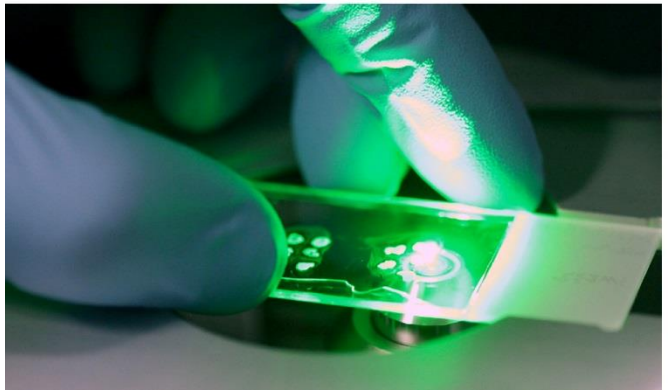




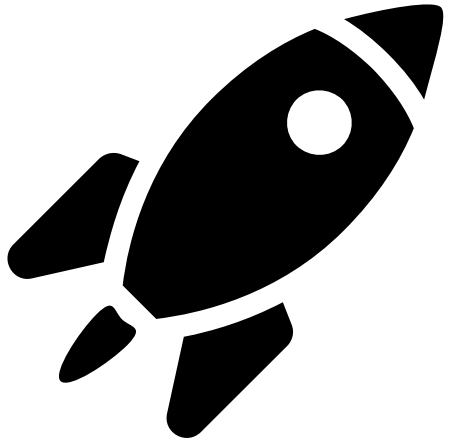
Disclosures

1. I am a Federal employee.
2. Income sources also include second opinion consultations and investments that comply with Federal Ethics rules.
3. My wife is an employee of Baltimore County and earns income from Johns Hopkins Hospital.
4. I will not discuss unapproved uses of medications, foods, or devices.

Objectives

1. Overview of the clinical trial process
2. Engaging participants
3. Clinical trials language

Buckle up, this rocket goes fast…



Why Do Clinical Trials?

- Does it work? (BENEFIT)
  - Efficacy vs effectiveness
  - Control groups define the results
- Is it safe? (RISK)
  - Population-specific
  - Short-term vs long-term

Problems with Clinical Trials

- Bias!
  - Control group*
  - Blinded observations*
  - Explicit inclusion/exclusion criteria*
  - Representative study populations
  - Statistics to handle dropouts
  - Prespecified outcomes measures
  - Publication bias

Types of Clinical Studies

- Treatment
- Prevention
- Diagnostic
- Supportive Care
- Screening
- Health Services Research
- Basic Science
- Device Feasibility

Phases of Clinical Trials

Phase 1

Study Participants: 20 to 100 healthy volunteers or people with the disease/condition.

Length of Study: Several months

Purpose: Safety and dosage

Approximately 70% of drugs move to the next phase

Phases of Clinical Trials

Phase 2

Study Participants: Up to several hundred people with the disease/condition.

Length of Study: Several months to 2 years

Purpose: Efficacy and side effects

Approximately 33% of drugs move to the next phase

Phases of Clinical Trials

Phase 3

Study Participants: 300 to 3,000 volunteers who have the disease or condition

Length of Study: 1 to 4 years

Purpose: Efficacy and monitoring of adverse reactions

Approximately 25-30% of drugs move to the next phase

Phases of Clinical Trials

Phase 4

Study Participants: Several thousand volunteers who have the disease/condition

Purpose: Safety and efficacy

- Effectiveness studies
- Dissemination/implementation

Other Interventions

- Surgery
- Gene therapy/genetic modification*
- Devices
- Diets
- Behavior interventions
- Others

Clinical Trials Designs

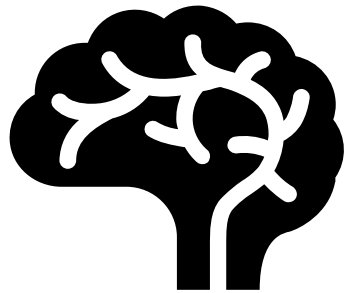
- Single Group: single arm
- Parallel: one of two or more groups in parallel for the duration of the study
- Crossover
- Factorial: two or more interventions, each alone and in combination evaluated in parallel against a control group
- Sequential: participants assigned to receive interventions based on prior milestones being reached in the study, such as in some dose escalation and adaptive design studies

Clinical Trials Designs

- Randomized double-blind placebo-controlled trials
- Post-marketing
- Comparative effectiveness
- Master protocols
  - Platform
  - Umbrella
  - Basket

Clinical Trials Designs

- Inclusion/exclusion criteria
- Number/complexity of outcome measures
  - Number of visits
  - Blood draw volumes
  - Scanner time
- Duration of follow-up
- Statistical power and analyses



Importance of Patient Advocacy Groups (PAGs)

- Involvement as early as possible/feasible!
- Critical for determining patient-relevant outcomes in natural history studies and clinical trials
- Required for some clinical research study applications

Consent Forms: Required Elements

- Voluntary Participation
- Purpose of Research
- Description of the Procedures
- Risks
- Confidentiality
- Potential Benefits
- Financial Considerations
- Withdrawal from Research
- Alternatives to Participation
- Explanation of Resources Available in Case of Injury
- Contact Information

National Institute of Neurological Disorders and Stroke

Source: NHGRI

ClinicalTrials.gov

NIH U.S. National Library of Medicine

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Before participating in a study, talk to your health care provider and learn about the risks and potential benefits.

Find a study (all fields optional)

Status

- ○ Recruiting and not yet recruiting studies
- ● All studies

Condition or disease (For example: breast cancer)

Other terms (For example: NCT number, drug name, investigator name)

Country

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Questions?

Thank you!

Thank you!

Thank you!

Thank you!

Thank you!

Thank you!

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