



# AN INTRODUCTION TO CLINICAL TRIALS

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# Why is Research Important?



Helps patients, physicians, and scientists gain important knowledge about the safety of new procedures, devices, or treatments



Leads to new methods of preventing, diagnosing, treating, and finding the cures for diseases

# What is Clinical Research?



**Research:** collecting and using information to find new answers to problems

**Clinical Research:** all research involving people



What are the goals of clinical research?

# Goals of Clinical Research

- Study the impacts, patterns, causes, and effects of health and disease across a group (“Epidemiology”)
- **For example:** A study looking at if exposure to a toxic site was related to a higher incidence of lupus



# Goals of Clinical Research



- The purpose of the study is to understand how patients' actions affect their health (“Behavioral”)



- **For example:** A study looking at whether or not lupus patients are more likely to wear sunscreen during the summer if a mobile app sends them daily reminders

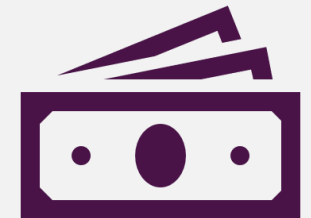
# Goals of Clinical Research



- Examines how patients access health care services, and how much health care services cost the patient and the health care system (“**Health Services**”)



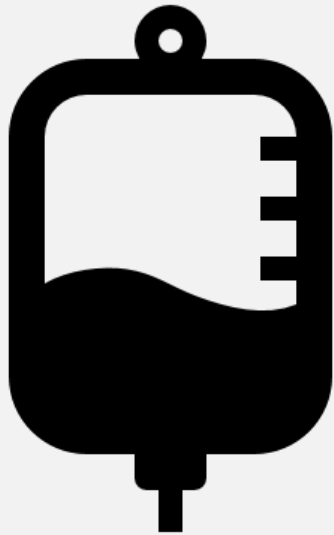
- **For example:** A study examining the costs of care for patients with lupus



# Goals of Clinical Research



- Evaluate effects of a specific treatment or medication on the people's health (“Clinical Trials”)



- **For example:** A study examining whether injection or infusion of the drug Belimumab is as effective for treating skin and joint lupus symptoms compared to the current standard-of-care treatment

# The goal of clinical trials is to:

- 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





# Who Can Participate in Clinical Trials?

- **Criteria-** Definition of a group that allows something to be studied (ex: SLE Diagnosis Type, Age, Sex)
  - Identifies a target population of individuals who are **similar** in a set of ways (e.g. age, sex, disease activity level)
- Each study has its own **inclusion criteria** to define who **can** participate in a trial and **why**
  - For example: One study may enroll patients who have lupus in their kidneys. Another study may enroll patients who have been diagnosed with lupus in the past year.



# Who Cannot Participate in Clinical Trials?

- Each study has its own exclusion criteria to define who **cannot** participate and why
- For example: exclusion of patients with renal lupus
  - Other reasons for exclusion:
    - Treatment may not be safe for individuals with certain other illnesses
    - A disease may either be too active or inactive to be able to test whether a new medication will work safely and effectively

# How do Clinical Trials Work?

A “**Protocol**” is a Roadmap for the Study

How long the study lasts

Information that is collected

An **Institutional Review Board (IRB)** checks this roadmap to make sure it is safe for patients

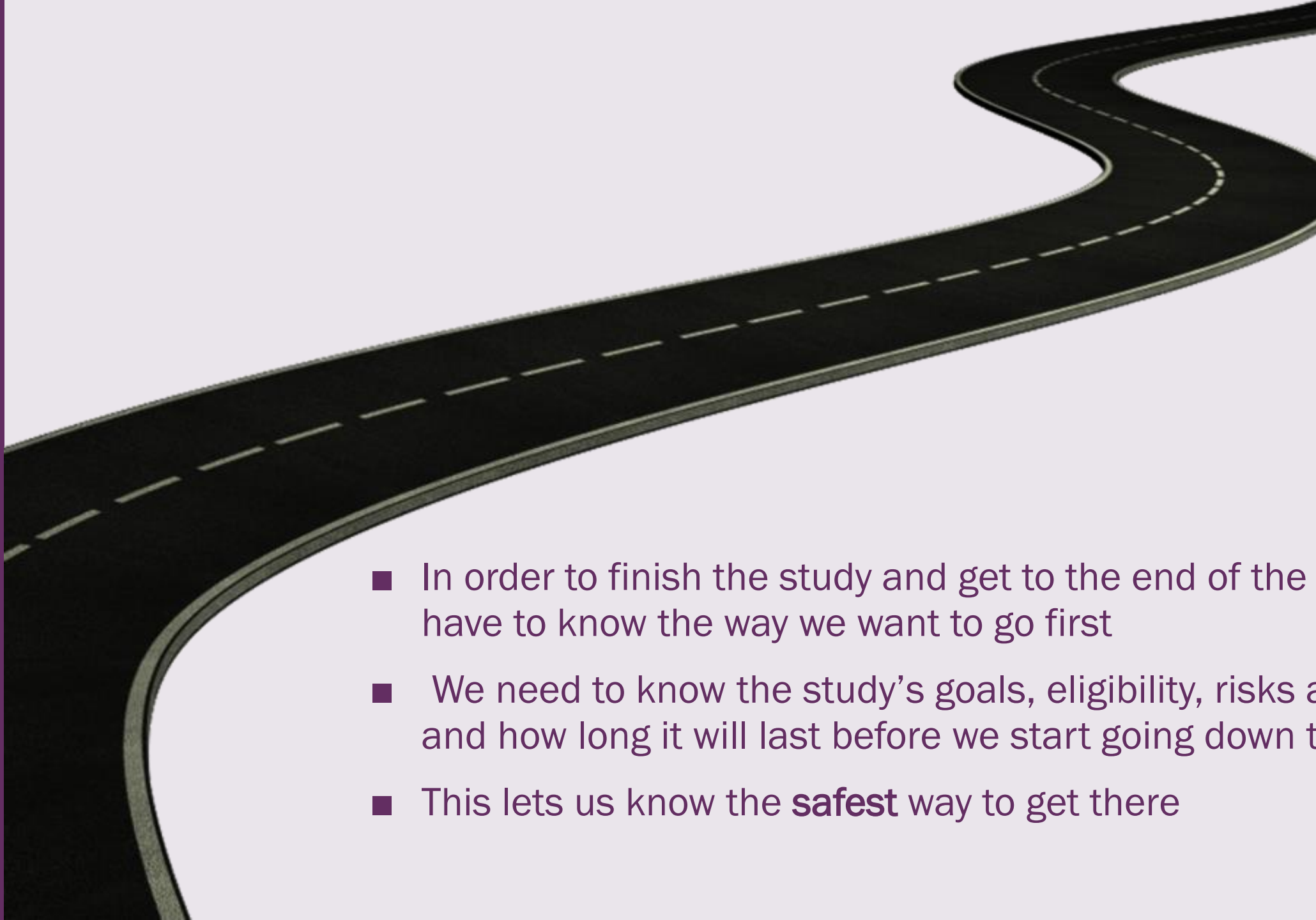
risk protection

study rules

eligibility

study goals





- In order to finish the study and get to the end of the road, we have to know the way we want to go first
- We need to know the study's goals, eligibility, risks and benefits, and how long it will last before we start going down the road
- This lets us know the **safest** way to get there



## The Belmont Report

Published in 1979 and established the guidelines for ethics in medical research

# Respect for Persons

- People have the ability to decide if they want to participate, not participate, or withdraw from a study
- People who are not able to consent because of age, illness, or mental disability must be protected
- For example, a child with lupus cannot participate in a study unless his/her parents or guardian consents





# Beneficence

- Research must make all efforts to maximize the benefits of the research and minimize risks
- For example, patients with severe lupus kidney disease were not able to participate in a study comparing the drug belimumab to standard lupus treatment
  - The initial data from the trial suggested that the “standard of care” for kidney lupus was better

# Justice

- Patients cannot be denied treatment because they don't want to participate in research
- Risks and benefits of the study must be described equally, and good AND bad results must be shared
- For example, if a patient is enrolled in a clinical trial comparing the effects of belimumab on lupus symptoms to a placebo, they should be told about the side effects AND the potential benefits of the drug





# Institutional Review Board (IRB)



- IRBs are committees that review the safety and ethics of research protocols. They are made of **community members** and are **independent** of the study.
- Each institution has its own IRB
- Make sure that all participants are treated **fairly, equally, and ethically**, and that benefits of each study outweigh risks

# Informed Consent

Taking part in a clinical trial is **OPTIONAL** and participants can withdraw at any time!

- **Informed Consent**- conversation that provides potential participants with information about the study to allow them to **choose** whether or not to participate
  - Member of the research team talks through the details of the study, answers questions, and provides a consent document to review with the person considering participating in research study.
  - They give the person time to review material and make their decision.



# 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”

# Example of informed consent document

## 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

# Example of informed consent document

## Informed Consent Document

- **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

# Example of informed consent document

## Informed Consent Document

- **Payment for participation**
  - How much will you be paid?
  - When will you be paid?
  - What do you have to do in the study to be paid?
- **Alternative Treatments**
  - Are there other ways to treat the condition being studied?
    - You must be told of these options if they exist
- **Payment for study-related injury**
  - 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

- ❖ The document will then 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

# 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**


# Example of informed consent document

## 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





Questions? Thank you!

# Words to Remember

- **Epidemiology**- Study the impacts, patterns, causes, and effects of health and disease across a group
- **Behavioral**- aims to better understand a person's actions on his/her health
- **Health Services**- Examines how patients access health care services, and how much health care services cost the patient and the health care system
- **Clinical Trials**- Evaluate effects of a specific treatment or medication on the people's health
- **Criteria**- Definition of a group that allows something to be studied (ex: SLE Diagnosis Type, Age, Sex)
- **Inclusion criteria**- list that defines who **can** participate in a trial and why
- **Exclusion criteria**- list that defines who **cannot** participate in a trial and why
- **Active SLE**- Lupus symptoms are worse than normal and patient is having a lupus flare
- **Inactive SLE**- Lupus is in remission and patient is not having active lupus symptoms

# Funding and Acknowledgements

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