LUPUS RESEARCH AND CLINICAL TRIALS

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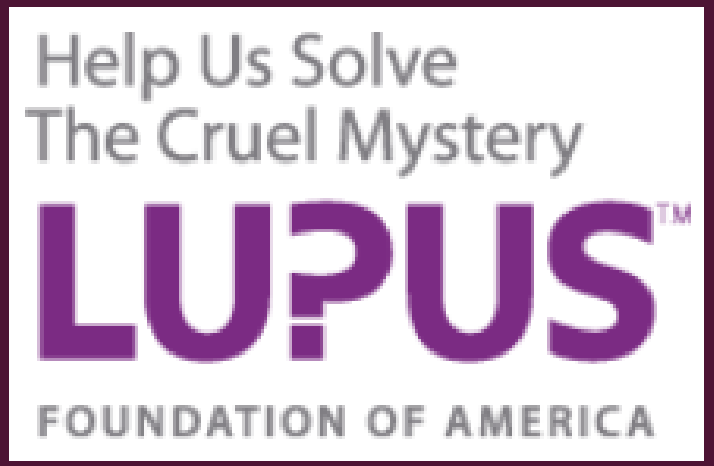

WHAT IS CLINICAL RESEARCH?

- Clinical research refers to studies in which people participate as patients or healthy volunteers.

- Different terms are used to describe clinical research, including:
  - Clinical studies
  - Clinical trials
  - Studies
  - Research
  - Trials
  - Protocols

- Clinical research may have a number of goals, such as:
  - Developing new treatments or medications
  - Identifying causes of illness
  - Studying trends
  - Evaluating ways in which genetics may be related to an illness

https://www.fda.gov/ForPatients/ClinicalTrials/default.htm

CLINICAL RESEARCH VERSUS MEDICAL TREATMENT

	Clinical Research	Medical Treatment
Intent	Answers specific questions through research involving numerous research volunteers.	Address the needs of individual patients.
Intended Benefit	Generally designed and intended to benefit future patients.	Intended to benefit the individual patient.
Funding	Paid for by drug developers and government agencies.	Funded by individual patients and their health plans.
Timeframe	Depends on the research protocol.	Requires real-time decisions.
Consent	Requires written informed consent.	May or may not require informed consent.
Assessment	Involves periodic and systematic assessment of patient data.	Based on as-needed patient assessment.
Protections	Protected by government agencies, institutional review boards, professional standards, informed consent, and legal standards.	Guided by state boards of medical practice, professional standards, peer review, informed consent, and legal standards.
Certainty	Tests products and procedures of unproven benefit to the patient.	Uses products and procedures accepted by the medical community as safe and effective.
Access to Information	Considered confidential intellectual property.	Available to the general public through product labeling.
Release of Findings	Published in medical journals, after clinical research ends.	Individual medical records are not released to the general public.

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WHAT IS A CLINICAL TRIAL?

- Clinical trials are studies that research medications, vaccines, devices or procedures to determine if they are safe and work in people who have diseases such as lupus.
- These studies may show which medical approaches work best for certain groups of people.
- People who participate in clinical trials are always volunteers.

https://www.fda.gov/ForPatients/ClinicalTrials/default.htm

Clinical Trials: What Patients Need to Know

en español

Learn more about clinical trials and find a trial that might be right for you. Clinical trials are voluntary research studies conducted in people and designed to answer specific questions about the safety or effectiveness of drugs, vaccines, other therapies, or new ways of using existing treatments. It is important to remember that the FDA does not conduct Clinical Trials.

Search for a Clinical Trial

Enter a word or phrase, such as the name of a medical condition or intervention.
Example: Cancer AND Los Angeles or expanded access AND compassionate use

Search

Learn More About Clinical Trials

WHO SHOULD PARTICIPATE IN CLINICAL TRIALS?

- Some people participate in clinical trials because none of the standard (approved) treatment options have worked, or they are unable to tolerate certain side effects.
- Clinical trials provide another option when standard therapy has failed.
- Others participate in trials because they want to contribute to the advancement of medical knowledge.

DIFFERENT TYPES OF CLINICAL TRIALS

- **Treatment Research** generally involves an intervention such as medication, psychotherapy, new devices, or new approaches to surgery or radiation therapy.
- **Prevention Research** looks for better ways to prevent disorders from developing or returning. Different kinds of prevention research may study medicines, vitamins, vaccines, minerals, or lifestyle changes.
- **Diagnostic Research** refers to the practice of looking for better ways to identify a particular disorder or condition.
- **Screening Research** aims to find the best ways to detect certain disorders or health conditions.
- **Quality of Life Research** explores ways to improve comfort and the quality of life for individuals with a chronic illness.
- **Genetic studies** aim to improve the prediction of disorders by identifying and understanding how genes and illnesses may be related. Research in this area may explore ways in which a person's genes make him or her more or less likely to develop a disorder. This may lead to development of tailor-made treatments based on a patient's genetic make-up.
- **Epidemiological studies** seek to identify the patterns, causes, and control of disorders in groups of people.

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CLINICAL TRIALS FOR NEW DRUGS

- Federal law requires that all drugs approved by the FDA be proven safe and effective in well-controlled clinical trials.
- After studies with animals, tissue, or cell cultures show that a substance has a promising therapeutic effect, a drug company may file an investigational new drug application with the FDA and, if approved, may begin clinical trials with human volunteers.

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PHASES OF CLINICAL TRIALS

- **Phase I**
  - Study Participants: 20 to 100 healthy volunteers or people with the disease/condition.
  - Length of Study: Several months
  - Purpose: Safety and dosage
    - Phase I studies are closely monitored and gather information about how a drug interacts with the human body. Researchers adjust dosing schemes based on animal data to find out how much of a drug the body can tolerate and what its acute side effects are.
  - Approximately 70% of drugs move to the next phase

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PHASES OF CLINICAL TRIALS

- **Phase II**
  - Study Participants: Up to several hundred people with the disease/condition.
  - Length of Study: Several months to 2 years
  - Purpose: Efficacy and side effects
    - These studies typically aren't large enough to show whether the drug will be beneficial. Instead, Phase 2 studies provide researchers with additional safety data. Researchers use these data to refine research questions, develop research methods, and design new Phase 3 research protocols.
  - Approximately 33% of drugs move to the next phase

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PHASES OF CLINICAL TRIALS

- **Phase III**
  - 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
    - Researchers design Phase 3 studies to demonstrate whether or not a product offers a treatment benefit to a specific population and provide most of the safety data. In previous studies, it is possible that less common side effects might have gone undetected. Because these studies are larger and longer in duration, the results are more likely to show long-term or rare side effects.
  - Approximately 25-30% of drugs move to the next phase

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PHASES OF CLINICAL TRIALS

- **Phase IV**
  - Study Participants: Several thousand volunteers who have the disease/condition
  - Purpose: Safety and efficacy
    - Phase 4 trials are carried out once the drug or device has been approved by FDA.

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ARE CLINICAL TRIALS SAFE?

- The Federal government has regulations and guidelines for clinical research to protect participants from unreasonable risks.
- Although efforts are made to control the risks to participants, some may be unavoidable because we are still learning more about the medical treatments in the study.

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INFORMED CONSENT

- Informed consent is the process of learning the key facts about a clinical trial before deciding whether or not to participate. It is also a continuing process throughout the study to provide information for participants.
- To help someone decide whether or not to participate, the doctors and nurses involved in the trial explain the details of the study.
- If the participant's native language is not English, translation assistance can be provided.
- To make an informed decision about whether to participate or not in a clinical trial, people need to be informed about:
  - what will be done to them,
  - how the protocol (plan of research) works,
  - what risks or discomforts they may experience,
  - participation being a voluntary decision on their part.
- Then the research team provides an informed consent document that includes these details about the study and the participant decides whether or not to sign the document.
- Informed consent is not a contract, and the participant may withdraw from the trial at any time.

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TERMS USED IN CLINICAL TRIALS

- Placebo
  - A placebo is a pill, liquid, or powder that has no treatment value. It is often called a sugar pill. In clinical trials, experimental drugs are often compared with placebos to evaluate the treatment's effectiveness.

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TERMS USED IN CLINICAL TRIALS

- Randomized
  - A randomized trial is one where patients are assigned by chance (like flipping a coin) to different groups and may receive different treatments.

TERMS USED IN CLINICAL TRIALS

- Blinded
  - If a trial is blinded, it means that the participant does not know what group or medication they are assigned to. In a double-blinded trial, neither the participant nor the investigator know what group or medication the patient is assigned to. Blinding minimizes bias.
- Open-Label
  - Participants know which group/medication they are assigned to.

WHO CAN PARTICIPATE IN CLINICAL TRIALS?

- All clinical trials have guidelines about who can participate. Before joining a clinical trial, a participant must qualify for the study.
- Researchers use inclusion/exclusion criteria to determine if someone is eligible to enroll in a clinical trial. The factors that allow someone to participate in a clinical trial are called "inclusion criteria" and those that disallow someone from participating are called "exclusion criteria".
- These criteria are based on such factors as age, gender, the type and stage of a disease, previous treatment history, and other medical conditions.
- Some research studies seek participants with illnesses or conditions to be studied in the clinical trial, while others need healthy participants.
- It is important to note that inclusion and exclusion criteria are not used to reject people personally. Instead the criteria are used to identify participants and help ensure that researchers will be able to answer the questions they plan to study.

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DIVERSITY IN CLINICAL TRIALS

- It is important to test drugs and medical products in the people they are meant to help.
- It is also important to conduct research in a variety of people, because different people may respond differently to treatments.
- Racial health disparities may stem from lack of access to quality healthcare and proper health awareness.
- This means in some cases, the incidence of disease may not match the trial population.

WHAT HAPPENS DURING A CLINICAL TRIAL?

- The clinical trial process depends on the kind of trial being conducted. Clinical trials also have a research team that is led by a research/study coordinator and may include doctors, nurses, social workers and other healthcare professionals. They check the health of the participant at the beginning of the trial, give specific instructions for participating in the trial, monitor the participant carefully during the trial, and stay in touch after the trial is completed.
- Some clinical trials involve more tests and doctor visits than the participant would normally have for an illness or condition. For all types of trials, the participant works with a research team. The clinical trial can only be successful if the protocol is carefully followed and there is frequent contact with the research staff.

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BENEFITS OF PARTICIPATING IN CLINICAL TRIALS

Clinical trials allow participants to:

- Play an active role in their own health care.
- Gain access to new research treatments before they are widely available.
- Obtain expert medical care during the trial.
- Receive compensation, in some instances, for their participation.
- Help others by contributing to medical research.

RISKS OF PARTICIPATING IN CLINICAL TRIALS

- There may be unpleasant, serious or even life-threatening side effects to experimental treatment.
- The experimental treatment may not be effective for the participant.
- The protocol may require more time and attention than would a non-protocol treatment, including trips to the study site, more treatments, hospital stays or complex dosage requirements.

HOW IS THE SAFETY OF THE PARTICIPANT PROTECTED?

- The ethical and legal codes that govern medical practice also apply to clinical trials. In addition, most clinical research is federally regulated with built-in safeguards to protect the participants. The trial follows a carefully designed protocol, a study plan which details what researchers will do in the study. As a clinical trial progresses, researchers report the results of the trial at scientific meetings, to medical journals, and to various government agencies. Individual participants' names will remain private and will not be mentioned in these reports.

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CAN I LEAVE A CLINICAL TRIAL AFTER IT HAS BEGUN?

- Yes. You can leave a clinical trial at any time.
- When withdrawing from the trial, you must let the research team know about it and your reasons for leaving the study.

THINGS TO CONSIDER BEFORE PARTICIPATING IN A CLINICAL TRIAL

- You should know as much as possible about the clinical trial and feel comfortable asking the members of the research team questions about it, the care expected while in a trial, and the cost, if any, of participating in the trial.
- The decision to participate in a clinical trial should not be taken lightly. If you would like to participate, you should have a clear understanding of the nature and aims of the study and your role in it. Ask the research team questions about anything that is unclear.

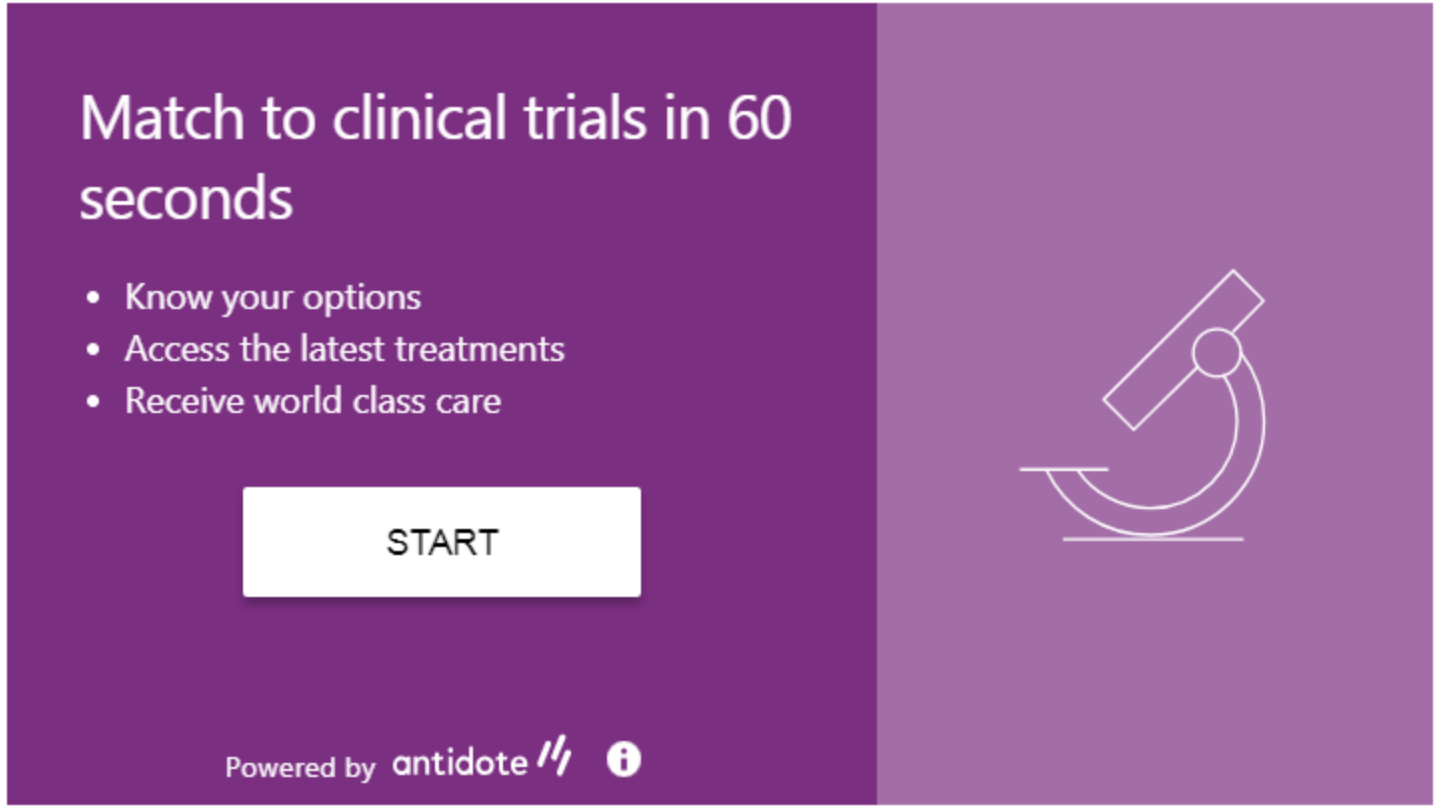
EXAMPLE QUESTIONS

- What is the purpose of the study?
- Why do researchers think the experimental treatment may be effective?
- Have other studies been done with this treatment and what were the findings?
- What kinds of test are involved?
- How do the possible risks, side effects, and benefits in the study compare with my current treatment?
- How long will the study take?
- Will there be any cost? Reimbursement?
- Who will be in charge of my care?

INFORMATION ON UPCOMING CLINICAL TRIALS

- For more information about clinical trials in your area, visit the National Resource Center on Lupus:
- **Resources.Lupus.org/Entry/Search-For-Clinical-Trials**

Search for clinical trials in your area



Search these sites to find information about clinical trials near you.

- **ClinicalTrials.gov** - a registry of both publicly and privately supported clinical studies maintained by the US National Institutes of Health.
- **CenterWatch.com** - the largest online listing of industry-sponsored global clinical trials.

NIH U.S. National Library of Medicine

ClinicalTrials.gov

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ClinicalTrials.gov is a database of privately and publicly funded clinical studies conducted around the world.

Explore 286,629 research studies in all 50 states and in 204 countries.

ClinicalTrials.gov is a resource provided by the U.S. National Library of Medicine.

IMPORTANT: Listing a study does not mean it has been evaluated by the U.S. Federal Government. Read our disclaimer for details.

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

Search | Advanced Search

OTHER WAYS YOU CAN PARTICIPATE IN LUPUS RESEARCH

- Share your perspective—Taking part in surveys, interviews, or focus groups is a great way to help shape and advance lupus research. Keep an eye out for upcoming opportunities to share your perspective via PARTNERS, a patient-powered research network focused on children and teens with rheumatic diseases and their families. Follow the Lupus Foundation of America on Facebook or Twitter for announcements.
- Join a registry—A medical registry is a database of key information about individuals with a specific condition. Researchers use registries to understand the factors that contribute to the development and progression of lupus. These registries often follow strict protocols (plans) as well as state and federal laws to secure your health information and protect your identity. Registries can also be used to find individuals who may be eligible to volunteer for clinical research.

THANK YOU & QUESTIONS??