Lupus and Clinical Trials

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Why should I participate in Clinical Trials?

- To gain a better understanding of effective treatment for lupus patients.
- To include diverse populations in research.
- To obtain data on how patients will react to the side-effects of the drug.
- To be more participatory in health care treatment.
- To gain access to new treatments before they are widely available.
- To help others by contributing to medical research studies.

Informed Consent

- Informed consent is the process of learning the key facts about a clinical trial before deciding whether or not to participate.
- Provides information for participants.
- Helps someone decide whether or not to participate.
- If the participant's native language is not English, translation assistance can be provided.

Source: www.clinicaltrials.gov

Informed Consent (continued)

- The research team then provides an informed consent document that includes:
- details about the study
- purpose
- duration
- required procedures
- key contacts
- risks and benefits
- The participant then 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.

source: www.clinicaltrials.gov

Clinical Trial Process

- The clinical trial process depends on the kind of trial being conducted
- The clinical trial team includes doctors and nurses as well as social workers and other health care professionals.
- The team checks the health of the participant at the beginning of the trial
- Gives specific instructions for participating in the trial
- Monitors the participant carefully during the trial
- Stays in touch after the trial is completed.

Source: www.clinicaltrials.gov

Clinical Trial Process(continued)

- 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.
- Clinical trial participation is most successful when the protocol is carefully followed and there is frequent contact with the research staff.

Source: www.clinicaltrials.gov

Types of Clinical Trials

- Treatment trials test experimental treatments, new combinations of drugs, or new approaches to surgery or radiation therapy.
- Prevention trials look for better ways to prevent disease in people who have never had the disease or to prevent a disease from returning. These approaches may include medicines, vaccines, vitamins, minerals, or lifestyle changes.
- Diagnostic trials are conducted to find better tests or procedures for diagnosing a particular disease or condition.
- Screening trials test the best way to detect certain diseases or health conditions.
- Quality of Life trials explore ways to improve comfort and the quality of life for individuals with a chronic illness.

Source: www.clinicaltrials.gov

Clinical Trial Terms

- **What "Randomized" Means**
  If you enroll in a "randomized" clinical trial, you will be randomly assigned to a treatment or to a control group that gets a placebo (inactive treatment).
- **What "Double-Blind" Means**
  If a study is "double-blind" neither the participant nor the people conducting the trial know which group you have been assigned to—the one with the test drug or the placebo (inactive treatment).
- **What "Placebo-Controlled" Means**
  The purpose of the placebo-control group is to better assess the good and bad effects of the experimental treatment.

Clinical Trial Types

- A clinical study is a research study that involves human volunteers, either patients or healthy volunteers. The U.S. National Institutes of Health defines the two main types of clinical studies: interventional and observational studies:
- **Clinical Trial (Interventional Study)**
  "In an interventional study, participants receive specific interventions according to the research plan or protocol created by the investigators.
- These interventions may be medical products, such as drugs or devices; procedures; or changes to participants' behavior, for example, diet."
- **Observational Study**
  "In an observational study, investigators assess health outcomes in groups of participants according to a protocol or research plan.
- Participants may receive interventions, which can include medical products, such as drugs or devices, or procedures as part of their routine medical care, but participants are not assigned to specific interventions by the investigator (as in a clinical trial). For example, investigators may observe a group of older adults to learn more about the effects of different lifestyles on cardiac health."

Clinical Trial Guidelines

- All clinical trials have guidelines about who can participate.
- Inclusion/exclusion criteria helps produce reliable results in clinical research.
- 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.
- Before joining a clinical trial, a participant must qualify for the study. Some research studies seek participants with illnesses or conditions to be studied in the clinical trial, while others need healthy participants.

Source: www.lupusfoundation.org

Clinical Trials Phases I-IV

- In Phase I trials, researchers test an experimental drug or treatment in a small group of people (20-80) for the first time to evaluate its safety, determine a safe dosage range, and identify side effects.
- In Phase II trials, the experimental study drug or treatment is given to a larger group of people (100-300) to see if it is effective and to further evaluate its safety.
- In Phase III trials, the experimental study drug or treatment is given to large groups of people (1,000-3,000) to confirm its effectiveness, monitor side effects, compare it to commonly used treatments, and collect information that will allow the experimental drug or treatment to be used safely.
- In Phase IV trials, post marketing studies delineate additional information including the drug's risks, benefits, and optimal use.

Source: www.lupusfoundation.org

Clinical Trial Sponsorship

- Clinical trials are sponsored or funded by a variety of organizations or individuals such as physicians, medical institutions, foundations, voluntary groups, and pharmaceutical companies, in addition to federal agencies such as the National Institutes of Health (NIH), the Department of Defense (DOD), and the Department of Veterans Affairs (VA).
- Trials can take place in a variety of locations, such as hospitals, universities, doctors' offices, or community clinics.

What questions should I ask about study participation?

- Be prepared:
  - Plan ahead and write down possible questions to ask.
  - Ask a friend or relative to come along for support and to hear the responses to the questions.
  - Bring a tape recorder to record the discussion to replay later.

Study Considerations

- People should know as much as possible about the clinical trial and feel comfortable asking the members of the health care team questions about it. The following questions might be helpful for the participant to discuss.
  - What is the purpose of the study?
  - Who is going to be in the study?
  - Why do researchers believe the experimental treatment being tested may be effective? Has it been tested before?
  - What kinds of tests and experimental treatments are involved?
  - How do the possible risks, side effects, and benefits in the study compare with my current treatment?
  - How might this trial affect my daily life?
  - How long will the trial last?
  - Will hospitalization be required?
  - Who will pay for the experimental treatment?
  - Will I be reimbursed for other expenses?
  - What type of long-term follow up care is part of this study?
  - How will I know that the experimental treatment is working? Will results of the trials be provided to me?
  - Who will be in charge of my care?

source: www.clinicaltrials.gov

Role of the Institutional Review Board

- Every clinical trial in the U.S. must be approved and monitored by an Institutional Review Board (IRB) to make sure the risks are as low as possible and are worth any potential benefits.
- An IRB is an independent committee of physicians, statisticians, community advocates, and others that ensures that a clinical trial is ethical and the rights of study participants are protected.
- All institutions that conduct or support biomedical research involving people must, by federal regulation, have an IRB that initially approves and periodically reviews the research.

source.www.lupusfoundation.org

FDA Drug Approval Process

- **How is a medicine approved by FDA?**
- Drug companies seeking approval to sell a drug in the United States must test it.
- The drug company or sponsor performs laboratory and animal tests to discover how the drug works and whether it's likely to be safe and work well in humans.
- A series of tests in humans is begun to determine whether the drug is safe when used to treat a disease and whether it provides a real health benefit.
- The company then sends FDA's Center for Drug Evaluation and Research (CDER) the data from these tests to prove the drug is safe and effective for its intended use.
- If this review establishes that a drug's health benefits outweigh its known risks, the drug is approved for sale.

source: www.fda.gov

Varied Issues in Trial Participation

- **Does a participant continue to work with a primary health care provider while in a trial?**
  - Yes. Most clinical trials provide short-term treatments related to a designated illness or condition, but do not provide extended or complete primary health care. By having the health care provider work with the research team, the participant can ensure that other medications or treatments will not conflict with the protocol.
- **What are side effects and adverse reactions?**
  - Side effects are any undesired actions or effects of the experimental drug or treatment. Negative or adverse effects may include headache, nausea, hair loss, skin irritation, or other physical problems. Experimental treatments must be evaluated for immediate and long-term side effects.

Risks vs. Benefits

- **What are the benefits and risks of participating in a clinical trial?**
  - **Benefits**
    Clinical trials that are well-designed are the best approach for eligible 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 at leading health care facilities during the trial.
    - Help others by contributing to medical research.
  - **Risks**
    There are risks to 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 of their time and attention than would a non-protocol treatment, including trips to the study site, more treatments, hospital stays or complex dosage requirements.

Patient Safety

- The ethical and legal codes that govern medical practice also apply to clinical trials.
- Most clinical research is federally regulated with built in safeguards to protect the participants.
- The trial follows a carefully controlled 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.

Clinical Trial Withdrawal

- **Can a participant leave a clinical trial after it has begun?**
  - Yes. A participant can leave a clinical trial at any time. When withdrawing from the trial, the participant should let the research team know about it, and the reasons for leaving the study.
- An investigator can also withdraw a patient from a study due to safety concerns.

Lupus Drugs

- Because lupus is different for every person, treatments and medications are prescribed based on individual needs.
- Medicines may include over-the-counter pain relievers and anti-inflammatory medicines.
- When internal organs are affected, stronger prescription drugs are prescribed to quiet the immune system and protect organs such as the kidneys, heart, and lungs from further attack.
- In 2011, the first lupus therapy in 50 years was approved with Benlysta (belimumab) Co-developed with Human Genome Sciences and GlaxoSmithKline.

source: http://lupusresearchinstitute.org/lupus-facts/lupus-treatments

Clinical Trial Example (Phase IV)

- **Research Study Summary:**
  A clinical study for patients with Systemic Lupus Erythematosus
- **Research Study Title:**
  A 5-Year Prospective Observational Registry to Assess Adverse Events of Interest and Effectiveness in Adults With Active, Autoantibody-Positive Systemic Lupus Erythematosus Treated With or Without BENLYSTA™ (Belimumab)
- **Purpose:** The purpose of this registry is to collect additional information regarding the side effects and effectiveness of BENLYSTA (belimumab) when given with other lupus medicines to adults with active systemic lupus erythematosus (SLE). Information will be collected on serious events that are not that common or may only be seen with long-term treatment. These events include death, cancers, serious infections and other infections of interest, and serious mental health problems. Information on the effectiveness of BENLYSTA will also be collected.
- **Gender:**
  Both Male and Female
- **Age:**
  18 and up
- **Overall Status:**
  Recruiting
- **Lead Sponsor:**
  Human Genome Sciences Inc., a GSK Company
- **Duration:**
  146 Months
- **Facility Type:**
  N/A

Inclusion/Exclusion Criteria Example

- **Inclusion Criteria:**
- Clinical diagnosis of active SLE.
- Autoantibody-positive.
- Current SLE treatment includes BENLYSTA and/or immunosuppressants (for example, azathioprine, methotrexate, cyclophosphamide, mycophenolate, and biologics).
- **Exclusion Criteria:**
- Have received treatment with an investigational agent within the past year.
- Are currently enrolled in a placebo-controlled BENLYSTA (belimumab) clinical trial or a continuation trial in which belimumab is used as an investigational agent.
- Have a history of BENLYSTA exposure, but are not currently receiving BENLYSTA.
- Current SLE treatment includes only an antimalarial (for example, hydroxychloroquine) or only steroids.

Clinical Trial Example Locations in Illinois

United States	Illinois	Chicago	60611	GSK Investigational Site US GSK Clinical Trials Call Center 877-379-3718 GSKClinicalSuppo rtHD@gsk.com
United States	Illinois	Chicago	60612	GSK Investigational Site US GSK Clinical Trials Call Center 877-379-3718 GSKClinicalSuppo rtHD@gsk.com
United States	Illinois	Morton Grove	60053	GSK Investigational Site US GSK Clinical Trials Call Center 877-379-3718 GSKClinicalSuppo rtHD@gsk.com

Clinical Trial Tips

- If you find a trial that interests you, learn more.
- Check out the official trial protocol for full details on the trial.
- Contact a trial coordinator to find out more about the trial.
- Look into studies on the www.clinicaltrials.gov website.

source: www.lupustrials.org

Lupus Research

- Lupus research is on the cusp of explosive growth. As with any disorder—especially a complex one like lupus that tends to show up in different parts of the body—it's smart to stay up-to-date on the findings of clinical trials and other research in lupus.
- Researchers around the world are studying lupus. To explore their findings, search PubMED.org a National Institutes of Health (NIH) site that compiles biomedical literature citations and abstracts.
- On the PubMED.org site, try searching such general terms as **lupus**, **systemic lupus erythematosus**, and **discoid lupus**, as well as more specific ones for a lupus complication (**lupus nephritis, lupus central nervous system**, **lupus anemia, lupus ophthalmology, lupus pregnancy**).

source: www.lupustrials.org

Questions?

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