

A woman with dark hair is sitting on a white stool in a futuristic, glowing environment. She is wearing a black dress and high heels. The background is a mix of blue and green light with circular patterns. A large circular inset in the upper center shows a close-up of her face, which is also glowing. The overall aesthetic is high-tech and vibrant.

UNDERSTANDING

CLINICAL  
TRIALS

THE ROLE OF A VOLUNTEER

# CLINICAL TRIALS

THE INS AND OUTS  
OF PARTICIPATING  
IN A CLINICAL STUDY

BY BRUCE GOLDFARB

*Have you ever wondered* how you can help in the fight for better lupus treatment? You don't have to have a medical degree to get involved in lupus research—you can advance treatment by participating in a clinical trial. Participating in research is essential for furthering the understanding of lupus and other diseases and, ultimately, leads to more effective treatments.

“The role of volunteers as partners in clinical research is crucial in the quest for knowledge that will improve the health of future generations. The health of millions of people has been improved because of the willingness of thousands of individuals to take part in clinical trials, which in turn have brought advances in medical research,” says Steven I. Katz, M.D., director of the National Institute of Arthritis and Musculoskeletal and Skin Diseases.

Of particular importance to readers of *Lupus Now* is that participating in clinical research can benefit someone with lupus, says Kenneth Getz, co-founder and board chair of the Center for Information and Study on Clinical Research Participation (CISCRP).

## COMING TO TERMS WITH CLINICAL RESEARCH

**Adverse event:** An unwanted side effect of a drug

**Baseline:** Measurements taken at the beginning of a study

**Blinding:** Efforts to prevent participants from knowing whether they are in the treatment group or the placebo group. In a single-blind design, the participant is unaware of the nature of a drug or intervention, but the research team may know. In a double-blind study, neither the participant nor the research team knows who is getting a drug and who is receiving placebo.

**Cohort:** A group of people with characteristics in common

**Control:** People with similar characteristics as those in the intervention group but who receive placebo or only their usual care

**Endpoint:** An overall outcome that the study is intended to measure

**Informed consent:** The process of learning the key facts about a clinical trial before deciding whether to participate

**Institutional review board:** A committee of physicians, statisticians, health care professionals, community advocates, and others that ensures that a clinical trial is ethical and protects the rights of participants. All clinical trials in the United States must be approved by an IRB.

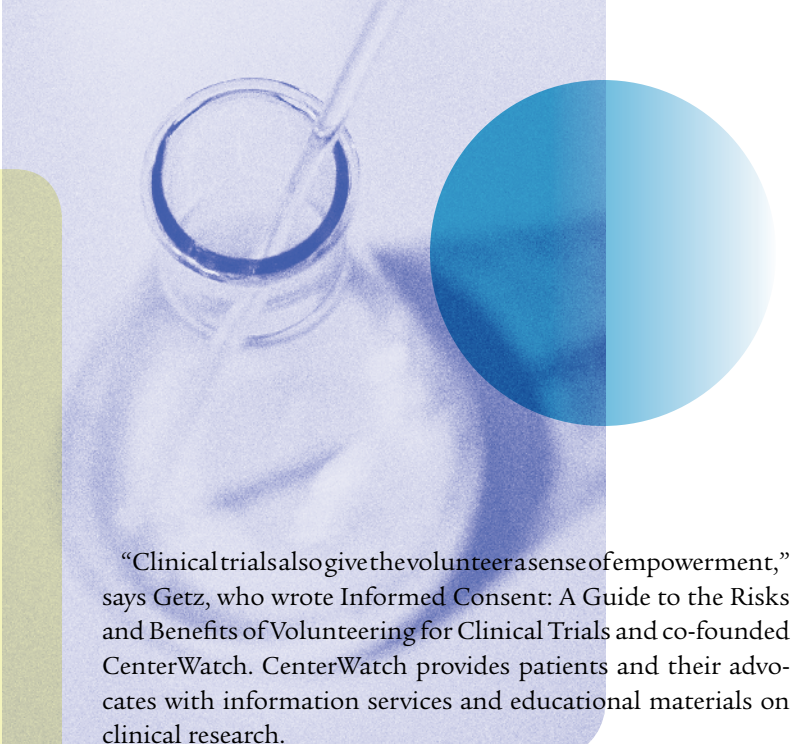
**Intervention:** Any drug or therapy that has an effect

**Placebo:** A pill, liquid, or powder that contains no active ingredient

**Protocol:** The design of a clinical trial

**Randomization:** The method by which participants are assigned to intervention and control groups, intended to reduce or eliminate biases

Source: [clinicaltrials.gov](http://clinicaltrials.gov), National Institutes of Health



“Clinical trials also give the volunteer a sense of empowerment,” says Getz, who wrote *Informed Consent: A Guide to the Risks and Benefits of Volunteering for Clinical Trials* and co-founded CenterWatch. CenterWatch provides patients and their advocates with information services and educational materials on clinical research.

“Throughout their participation, patients receive the benefit of interacting with very knowledgeable research professionals and experts,” Getz says. “Although their participation may not provide an effective treatment option for them directly, it will provide valuable medical information and insights that ultimately will benefit others facing the disease in the future.”

### Evaluating New Drugs

More than 80,000 clinical trials are conducted in the United States every year, according to Getz. Observational studies are designed to find trends in certain aspects of medical interest. Interventional studies test new drugs, devices, or therapies.

One of the most common types of study—and, some would argue, the most important—is a clinical trial to test new drugs.

Precious few drugs are available to treat systemic lupus erythematosus, and no new drugs for lupus have been approved by the Food and Drug Administration (FDA) in decades, according to Joan Merrill, M.D., of the Oklahoma Medical Research Foundation and medical director of the Lupus Foundation of America.

The good news is that there are at least 20 compounds in the pipeline with therapeutic potential for people with lupus, Merrill says.

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.

Phase I trials are usually conducted with small groups of people who may be healthy or have mild forms of the condition for which the drug is being tested. These trials evaluate the safety of a drug, determine a safe dosage range, and identify side effect.

ROSS ANANIA/GETTY IMAGES

**“The participant needs to be an active part of the study team, doing their best to stick to the protocol, keep their appointments, and promptly report any changes in their medical condition.”**

Drugs that pass phase I go on to phase II, which involves testing a drug in larger groups of people with the same illness to see if the drug is effective and to further monitor safety.

In phase III clinical trials, a drug is given to very large groups of people with the disease to determine its effectiveness, gauge how it compares with existing therapy, and collect information that will allow it to be used safely.

In each of these phases, there are two groups of participants: a group that receives the drug and a group that receives a placebo instead of the drug. A placebo, sometimes called a “sugar pill,” is a substance that does not have the active ingredient being studied. Depending upon the design of the study, a person will be randomly assigned to the control or placebo group.

But although those in the placebo group are not receiving the potential benefits of the drug, there still are advantages for them to take part in the study, says Ellen M. Ginzler, M.D., M.P.H., professor of medicine and chief of rheumatology at the State University of New York Downstate Medical Center, Brooklyn.

“They get more frequent follow-up, a thorough medical exam, and ready access to the medical professionals running the study,” she explains.

In some cases, drug companies will then conduct phase IV clinical trials, also known as post-marketing surveillance or open label studies. These studies collect additional information about the long-term risks, benefits, and use of newly approved drugs.

### **Treatment and Control**

Participation in a clinical trial isn't for everybody. An ideal candidate for a clinical trial is somebody “who fully understands what the trial involves, and the possible risks and benefits to them,” says Merrill.

A clinical trial candidate “should have a level of symptoms or illness that is appropriate for the kinds of treatments and options that the trial protocol will provide,” she adds. “The participant needs to be an active part of the study team, doing their best to stick to the protocol, keep their appointments, and promptly report any changes in their medical condition.”

## **QUESTIONS TO ASK**

The decision to participate in a clinical study should not be taken lightly. If you would like to participate, you should have a clear understanding of the nature and aims of a study and your role in it. Ask the research team questions about anything that is unclear.

Points to consider before agreeing to participate:

- The purpose of the study
- Your rights as a participant, including your right not to participate or to drop out at any time
- Your duties and obligations as a study participant
- How long the study will last and how much of a time commitment will be expected
- Any possible effects of the study, including potential benefits and risks
- Whether you will be reimbursed for expenses

If it is a study with an intervention—any drug or therapy that produces an effect—you should also be given information about the following:

- Your chances of being in the intervention group versus the control group
- How you will be assigned to one group or the other (this is often done by randomization)
- The chance of an adverse reaction or other risks from being in the intervention group
- Alternatives that you may want to consider rather than participating in a clinical study

Sources: [clinicaltrials.gov](http://clinicaltrials.gov); “Participating in medical research studies.” JAMA 2001; 285:686.

## TYPES OF CLINICAL STUDIES

Many types of clinical trials rely upon voluntary participation. Most clinical studies, but not all, test new drugs.

Types of clinical studies include:

**Treatment trials:** These trials test new therapies, devices, combinations of drugs, or approaches to treatment.

**Prevention trials:** These trials look for better ways to prevent disease, lessen its severity, or avoid the complications associated with a disease.

**Diagnostic trials:** These trials attempt to find better tests or procedures for diagnosing a disease or condition.

**Screening trials:** These trials test the best ways to detect certain diseases or health conditions.

**Quality-of-life trials:** These trials evaluate ways to improve comfort and quality of life for people with chronic illness.

Source: [clinicaltrials.gov](http://clinicaltrials.gov)

key facts of the study and answers any and all questions before a person agrees to participate. It is important to ask any questions you may have about the study and your role in it. The research team should be able to provide answers to your satisfaction. If you aren't sure of something, ask the team member for clarification. Many people also find it helpful to bring along a family member or caregiver to meet the research team.

Clinical study participants typically receive a thorough medical evaluation and, once accepted, may get access to new therapies that are not widely available. On the other hand, people who volunteer for a clinical trial must understand that they might receive no personal benefit from the experience, says Ginzler.

The best reason for participating in a clinical study is that research demands it. Only through voluntary participation will progress be made in treating lupus and preventing its complications. ■

This article is available in Spanish on our Web site, [lupus.org](http://lupus.org), in the En Español section, or by writing to [lupusnow@lupus.org](mailto:lupusnow@lupus.org).

Because lupus is more common, and can be more severe, in people of color, it is especially important that lupus studies contain ethnically and culturally diverse populations.

Researchers today collaborate with a variety of medical centers and research institutions in their own and other countries. "Multicenter and multinational trials are intended to provide a mix of people," says Ginzler, whose team is one of 30 centers in the United States and overseas that belong to the Systemic Lupus International Collaborating Clinics group.

Typically, there are specific criteria for inclusion in the trial. Researchers may be looking for people with milder, uncomplicated forms of lupus, for example, or people with family members who are also affected by lupus or other autoimmune diseases.

These details—and all other information essential to a clinical trial—are explained to potential study participants prior to enrollment, during the informed consent period. Informed consent is a process during which the research team discusses

## FOR MORE INFORMATION

**Clinicaltrials.gov**—Web site provided by the National Institutes of Health (NIH) with a searchable database of all NIH-sponsored clinical trials. Also has a list of frequently asked questions and other resources for people considering participation.

**CISCRP.org**—The Center for Information and Study on Clinical Research Participation. A nonprofit organization dedicated to providing information about clinical research participation.

**CenterWatch.com**—Web site that links together researchers and people interested in participating in clinical studies. Has information, resources, and a searchable database of trials.

**Taking Part in Research Studies: What Questions Should I Ask?**—Free brochure available from the National Prevention Information Network of the Centers for Disease Control and Prevention (CDC). Available at [www.cdcnpin.org/Brochures/ResStudy.pdf](http://www.cdcnpin.org/Brochures/ResStudy.pdf) or by calling the CDC at (800) 458-5231.



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