10 Things to Know About Clinical Trials

Clinical trials have led to important medical discoveries like vaccines and treatments for cancer, heart disease, diabetes, and rare diseases. Without clinical trials, we wouldn't have any of the medicines we take every single day.

Knowing more about clinical trials can help you feel more comfortable when deciding whether to take part in one. This resource offers 10 important things to understand about clinical trials so that you will be better prepared to start the conversation with your doctor and your family.

1. **A clinical trial is a research study involving people.** A clinical trial is designed to carefully assess the safety and effectiveness of an investigational drug, device or method of treatment that has not yet been approved by a regulatory agency such as the U.S. Food and Drug Administration (FDA) or the European Medicines Agency (EMA).

   Carefully conducted clinical trials determine if a treatment works without too many side effects. Without the patients who volunteer to participate in clinical trials, the development of new medicines would not be possible.

2. **The idea for a clinical trial often starts in the lab.** By the time a potential new medicine makes it to a clinical trial in which you might be eligible to participate, it has typically gone through at least three years of preclinical testing to see if the treatment is likely to be safe and effective for use in humans. Then, clinical trials in humans must be conducted before a drug can be approved. Clinical trials are divided into three phases and as the therapy moves through each phase the number of volunteers grows, and more information is gained about the treatment, its risks, and its effectiveness.

3. **Medical discoveries are not possible without the participation of volunteers.** Volunteers of all disease types and stages including newly diagnosed patients, patients in remission, patients with no other treatment options, and even healthy participants are needed for clinical trials. Some clinical trials are meant for those who have exhausted all treatment options, while others are meant to test ways to prevent recurrence. Some are designed for healthy participants to test ways to prevent the illness in the first place.

   It's important for individuals of varied races, ethnicities, ages, gender and sexual orientation to participate in clinical trials. Inclusion of participants from diverse backgrounds helps find better ways to fight diseases that disproportionately impact some populations.

4. **All clinical trials have guidelines about who can participate.** Before joining a clinical trial, a volunteer must qualify for the study. Examples of qualification criteria can include age, gender, the type and stage of a disease, previous treatment history, and other medical conditions. These guidelines are used to identify appropriate participants, promote participants' safety, and ensure that researchers learn all they need to know about the treatment, its risks and effectiveness.

5. **Clinical trials are tightly regulated to ensure maximum safety for participants.** The drug development process is strictly governed by regulatory agencies such as the FDA, EMA and the Medicines and Healthcare Products Regulatory Agency, or MHRA, in the United Kingdom to ensure that new drugs will be safe, effective and have the most benefit for patients. In the U.S., all clinical trials must be approved by an Institutional Review Board (IRB) which is made up of physicians, researchers and members of the local community. Similarly, in Europe and the UK the process is overseen by ethics committees, all with the responsibility to make sure that safety standards and processes are followed, and that risks are minimized in relation to potential benefits.

6. **Some common myths might keep people from learning more about clinical trials.** Misconceptions such as volunteers are merely guinea pigs or you may receive a "sugar pill" or placebo instead of a real drug are frequently stated reasons that may keep people from learning more about or participating in clinical trials. However, regulatory agencies set strict guidelines to ensure clinical trial volunteers are treated fairly and ethically. These ethical considerations also guide researcher's decisions during the trial. For example, a placebo may not be used in a clinical trial as it may be deemed unethical particularly in situations where patients have serious or life-threatening diseases. In these instances patients may instead receive the best available treatment (called "standard of care").

7. **Participation is always voluntary.** As with any medical treatment, participation in a clinical trial offers benefits and risks. To help you make an informed decision, the clinical study team is required to tell you about all known risks, benefits and available alternative health care options. This is called informed consent and is an ongoing, interactive discussion to help patients make decisions about whether to begin or continue to participate in the clinical trial. Participation is voluntary, and you always have the right to end participation at any time, even if the study is not complete.

8. **By volunteering for a clinical trial, you can play a more active role in health care innovation.** Participation in a clinical trial often provides patients access to potential new treatments and expert health care at leading institutions. By participating, you play an active role in your health and the health of others by contributing to scientific knowledge that may lead to innovative medical treatments for future patients.

9. **Clinical trials may have unique considerations for rare diseases.** Challenges exist for clinical trials in rare diseases including a small and geographically diverse patient population. Researchers and the FDA recognize the issues and are working to help make clinical trials more flexible and responsive to the needs of rare disease patients. Examples can include allowing treatment to move through development with a smaller number of patients, providing flexibility in the study visit schedule, arranging travel (for patients and caregivers) and at-home support for study procedures and/or drug administration.

10. **A patient with a rare disease can play a proactive role in finding information about clinical trials.** According to Global Genes, patients can help move drug discovery and pre-clinical development for rare diseases forward by taking part in patient registries, donating tissue to biobanks and sharing genetic data[1]. These types of patient registries help improve understanding and expand the knowledge base for companies developing treatments.

Only through clinical research can we discover new and better treatment options. Speak with your health care team or visit the website resources to learn more about how you or a loved one can play a role in current and future health care innovation.

Resources

Rare Diseases Clinical Research Network

ClinicalTrials.gov

TrialsToday.org

Orpha.net

World Health Organization International Clinical Trials Registry Platform

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Source:

1. Global Genes, Rare Toolkits: From Molecules to Medicine:
How are new drugs and therapies developed?
Available at: *globalgenes.org/toolkits/drug-development-overview/drug-development-process/*