Making the Decision to Enroll in a Clinical Trial

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Outline

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1 Introduction

- **What are clinical trials?**

Too much of the time, the perceptions about clinical trials are based on outdated assumptions and ideas. These assumptions can come from doctors as well as patients. Clinical trials are not random, unregulated experiments done on unwilling patients. They are tightly regulated with specific criteria for gathering data and most importantly, protect the patient participating in the clinical trial. Put another way, NO part of the clinical trial from patient questions to administration of experimental drugs is done without the patient's knowledge and consent.

- Your care and treatment are tightly monitored and controlled. No part of the trial is left to chance. The medical team conducting the trial will inform you about every possible outcome and they'll explain everything that is occurring. Finally, you are free to stop the trial at any time. You cannot be forced to continue if you don't want to. All you have to do is say, "I want to stop". You should also know that clinical trials are NOT treatments of last resort. You can request information about clinical trials, and presuming you meet the trial criteria, you can participate in a clinical trial at any point in your cancer journey. You do not have to wait until you've exhausted all other treatment options. You can join a trial at any time, and you can quit the trial whenever you want.

- Your oncologist may be reluctant to recommend a clinical trial, but there really isn't a legitimate reason for not considering a clinical trial, provided you meet the criteria for participating. Meeting the criteria is essential for making sure the medication can work for you. Otherwise, it may not help you. If your oncologist won't help you look for a clinical trial, then you might consider finding another oncologist. Their emphasis should be on getting you the best care and finding the best possible outcome. If they won't do that, you should find someone who will.

- Finally, you need to understand that clinical trials are not magic wands or miracle cures. But they are viable, legitimate treatment options that you can consider, regardless of the type of care you're getting now, no matter where you are on your cancer journey.

- Clinical trials serve as the foundation for most medical advances. Without clinical trials, many of the medical treatments and cures would not exist. Clinical trials offer hope to chondrosarcoma patients and a chance to help researchers and clinicians find more helpful treatments. The goal of a clinical trial is to determine if interventions are safe and effective. Clinical trials look at new ways to prevent, detect, or treat disease. This publication will introduce the elements involved in clinical trials to inform and help you make the decision to enroll in a clinical trial.

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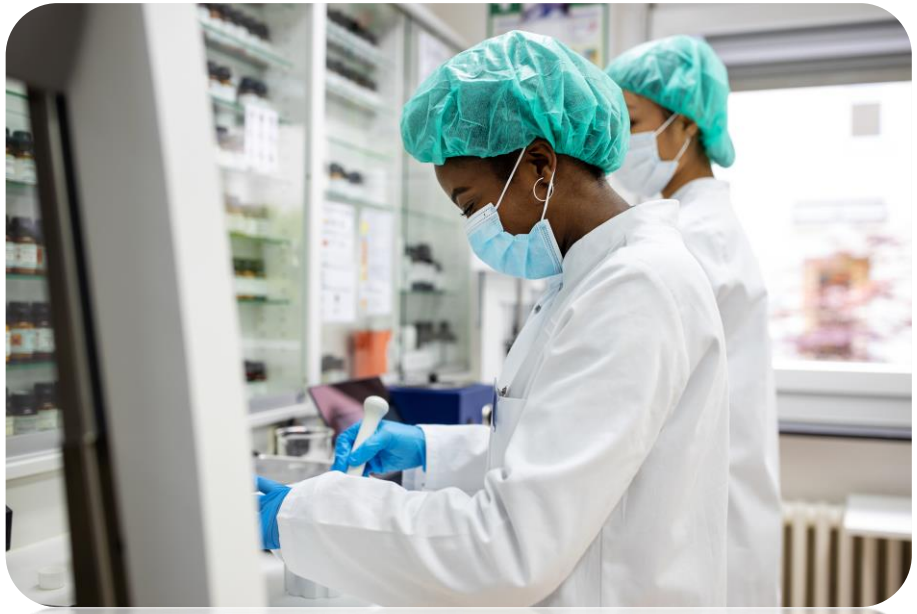
2 How does the research process work?

- The idea for a clinical trial often starts in the lab. After researchers test new treatments or procedures in the lab and in animals, the most promising treatments are moved into clinical trials. As new treatments move through a series of steps called phases, more information is gained about the treatment, its risks, and its benefits.
- A clinical trial is led by a principal investigator (PI). Members of the research team regularly monitor the patients' health to determine the study's safety and effectiveness.
- Participating in a clinical trial may take more time than standard treatment, and you may have more tests and treatments than you would if you were not participating in a clinical trial.
- The study team also may ask you to keep a log of symptoms or other health measures, fill out forms about how you feel, or complete other tasks. You may also need to travel or reside away from home to take part in a study.



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3 What are the phases of clinical trials?

Clinical trials are conducted in a series of stages called “phases.” Each phase has a different purpose and helps researchers address different questions.

Phase I trials: Researchers test a medication or treatment with a small group of people (approximately 20–80 patients) for the first time. The purpose is to study the drug or treatment to learn about safety and identify any side effects.

Phase II trials: The new medication or treatment is given to a larger group of people (100–300 patients) to determine its effectiveness and to further study its safety, risks, and benefits.

Phase III trials: The new medication or treatment is given to large groups of people (800 or more patients) to confirm its effectiveness, monitor side effects, compare it with standard or similar treatments, and collect information that will allow the new drug or treatment to be used safely.

Phase IV trials: After a drug is approved by the Food and Drug Administration: FDA and made available to the public, researchers track its safety in the general population, seeking more information about the medications or treatment’s benefits and use.

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4 What are the clinical trials protocols?

Clinical trials follow a plan known as a **protocol.** The protocol is carefully designed to balance the potential benefits and risks to participants and answer specific research questions. The study protocol describes:

- The goals of the study
- Who is eligible to take part in the clinical trial
- Protections against risks to participants
- Details about tests, procedures, and treatments
- The duration of the clinical trial
- What information / data will be gathered

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5 What is an Institutional Review Board?

- Clinical trials are carefully reviewed, approved, and monitored by an **Institutional Review Board (IRB)** to ensure that the risks are reduced and are outweighed by potential benefits.
- An IRB is an **independent committee** that consists of sarcoma specialists, oncologists, physicians, statisticians, and members of the community (sometimes surviving patients) who ensure that clinical trials are ethical and that the rights and safety of participants are protected both before the research starts and as it proceeds.
- You should ask the sponsor or research coordinator whether the trial you are thinking about joining is monitored and was approved by an IRB. You also have the right to ask questions or report any concerns or complaints to the IRB and contact information should be included in the informed consent document.

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6 What is informed consent?

- **Informed consent** is the process of providing you with all the key information about a research study before you decide whether to accept the offer to participate.

It is very important that you fully understand the terms, conditions, risks, benefits and any related covered and/or out of pocket costs of the clinical trial including travel expenses.

- Taking part in a clinical trial is voluntary and you can leave the study at any time. To participate, it will be necessary for you to sign an informed consent document. To help you decide whether to take part in the clinical trial, the staff of the research team are responsible for explaining all the details of the study and making sure you understand the research process as well as the terms and conditions of the study. If you do not read or understand English, a translator or interpreter should be provided for you.

- The research team provides an informed consent document that includes details about the study, such as its purpose, how long it's expected to last, tests or procedures that will be done as part of the research, and who to contact for further information. The informed consent document also explains the risks and potential benefits. The document outlines the costs that are covered by the study and any out-of-pocket costs required by you or your insurance, including travel expenses. Once you understand all the conditions included in the document, you can then decide whether to participate and sign the document.

If you do not understand everything contained in the informed consent, contact the person(s) responsible for explaining the clinical trial before you sign the document.

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7A What do the terms placebo, randomization, and blinded mean in clinical trials?

- **What is a Placebo?** Clinical trials that compare a new medication, new product, or new procedure with another that already exists, try to determine if the new one is better than the existing one or no treatment at all. In these types of studies, you may be assigned to receive a placebo. A placebo is a “sugar pill” that resembles medication or product being tested but has no treatment value. In research and clinical trials, comparing a new medication or product with a placebo can be the fastest and most reliable way to show if the medication is effective. However, there is some controversy and debate when placebos are used if you could be put at risk, specifically with an aggressive sub-type of chondrosarcoma. It is the responsibility of the research team to inform you if placebos are used in the study before entering a trial.

Clinical Trial participants may receive a placebo to compare results on the risks and benefits of a medication and learn from their differences.

- **What is Randomization?** Randomization is the process by which treatments are assigned to participants by chance rather than by choice. This is done to avoid any bias in assigning clinical trial participants to get one treatment over another. The effects of each treatment are compared at specific points during a trial. If one treatment is found superior, the trial is stopped so that most of the clinical trial participants receive the more beneficial treatment.

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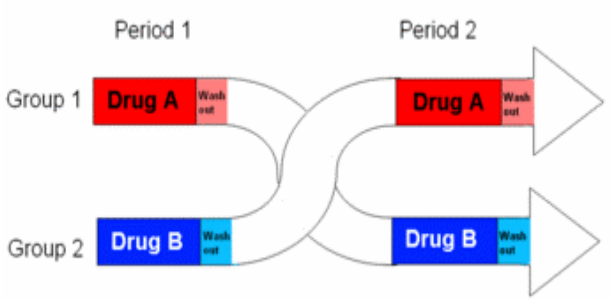
7B What do the terms placebo, randomization, and blinded mean in clinical trials?

- **What is a Blinded study?** Blinded studies are designed to prevent members of the research team and clinical trial participants from influencing the results. Blinding allows the collection of scientific data. In a single-blind study, you are not told what is being given, but the research team knows. In a double-blind study, neither you nor the research team are told what you are given; only the pharmacist knows. Members of the research team are not told which participants are receiving which treatment in order to reduce bias. If it becomes medically necessary, however, it is always possible to find out which treatment you are receiving.

- **Unblinding and Cross-over.** In a blinded study all participants are monitored to determine if the medication or product being studied is safe and effective. If participants in the clinical trial who are being monitored show no improvement or that chondrosarcoma has grown or spread, there is a period (4-6 weeks after the first treatment) that the researchers will unblind the study to determine if you received the medication or a placebo. If you receive a placebo, you would have the option to take the medication being studied and continue with the trial. If you were on the medication resulting in a poor outcome, you will be released from the study.



8 What do I need to know if I am thinking about taking part in a clinical trial?

Clinical trials involve medications, devices, or treatments designed to prevent or treat cancer and diseases like chondrosarcoma. Although these studies may provide direct benefit to patients, the main objective is to prove, by scientific means, the effects, and limitations of the experimental treatment.

A clinical trial treatment or outcome may result in helping patients directly; however, sometimes participants will not receive any direct benefit. If this is the case, study results can still serve as building blocks that are used to further research and help people later.

- **Inclusion and Exclusion Criteria:** Researchers follow clinical trial guidelines when deciding who can participate. Features that allow you to take part in a clinical trial are called "inclusion criteria." Those that exclude or prevent participation are "exclusion criteria." These criteria are based on elements such as age, gender, the type and stage of a disease, treatment history, and other medical conditions. Before joining a clinical trial, you must provide information that allows the research team to determine if you can take part in the study safely. Some research studies seek participants with illnesses or conditions to be studied in the clinical trial, while others need healthy volunteers. Inclusion and exclusion criteria are not used to reject people personally. Instead, the criteria are used to identify appropriate participants, keep them safe, and help ensure that researchers can find new information they need.

- Variables like how much of your time is needed, discomfort you may feel, or risk involved depend on the trial. While some require minimal amounts of time and effort, other studies may require a major commitment of your time and effort and may involve some discomfort. The research procedure(s) may also carry some risk. The informed consent process includes a detailed discussion of the study's procedures and tests and their risks.

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9 Risks of Clinical Trials

The **potential risks and side effects** associated with the study protocol are described in detail in the informed consent document, which participants are asked to consider and sign before participating in research. A member of the research team is required to explain the study and answer any questions about the study. Before deciding to participate, carefully consider risks and possible benefits.

Clinical trials may involve risk, as can routine medical care and the activities of daily living. When weighing the risks of research, think about these important issues:

- The possible harms that could result from taking part in the clinical trial.
- The chance and level of harm from side effects of the clinical trial.
- There may be unpleasant, serious, or even life-threatening effects of experimental treatment.
- The study may require more time and attention than standard treatment would, including visits to the study site, more blood tests, more procedures, hospital stays, or complex dosage schedules.

Most clinical trials pose the risk of minor discomfort, which lasts only a short time. Researchers try to limit patient discomfort during clinical trials. However, in some cases, participants have complications that require medical attention. In rare cases, participants have been seriously injured or have died of complications resulting from their participation in trials of experimental treatments.

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10 Potential benefits of Clinical Trials

Well-designed and well-executed clinical trials provide the best approach for you to:

- Gain access to new research treatments before they are widely available.
- Receive regular and careful medical attention from a research team that includes doctors and other health professionals.
- Treatment with study medications that may not be available elsewhere.
- Care from health care professionals who are familiar with the most advanced treatments available.
- The opportunity to learn more about an illness and how to manage it.
- Play an active role in your health care.
- Help others by contributing to medical research.

Clinical trials can provide many benefits to participants and society. However, before volunteering for a clinical trial, you should talk with your oncologist and the study team about the risks and benefits.

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11 Top 10 questions to ask about a clinical trial?

If you are thinking about taking part in a clinical trial, you should feel free to ask any questions or bring up any issues concerning the trial at any time. The following suggestions may give you some ideas as you think about your own questions.

#10 What is the purpose of the study? Why do researchers think the medication or product may be effective?

#9 How long will the study last?

#8 What is required and what are my responsibilities if I take part?

#7 What are the potential short-term, long-term benefits and risks?

#6 Are there any side effects?

#5 What kinds of therapies, procedures and/or tests will I have during the trial? Are any of them painful or require a period of recovery and if so, for how long?

#4 Will I be able to take my regular medications while taking part in the clinical trial or are there restrictions?

#3 Will I have to pay for any part of the trial such as tests or the study drug? If so, what will the charges likely be?

#2 Will my health insurance be likely to cover out of pocket costs? Who can help answer any questions from my insurance company or health plan?

#1 Will there be any travel or childcare costs that I need to consider while I am in the trial?

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12 What happens after a clinical trial is completed?

- After a clinical trial is completed, the researchers carefully examine information collected during the study before making decisions about the meaning of the findings and about the need for further testing.
- After a phase I or II trial, the researchers decide whether to move on to the next phase or to stop testing the treatment or procedure because it was unsafe or not effective. When a phase III trial is completed, the researchers examine the information and decide whether the results have medical importance.
- Results from clinical trials are often published in peer-reviewed scientific journals. If the results are particularly important, they may be featured in the news, and discussed at scientific meetings and by patient advocacy groups before or after they are published in a scientific journal.
- Once a new approach has been proven safe and effective in a clinical trial, it may become a new standard of medical practice. Published study results are available by searching for the study's official name or Protocol ID number in the National Library of Medicine's PubMed® database.

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13 What is the next step after I find a clinical trial?

To learn more about a specific clinical trial, contact the study coordinator or principal investigator of the clinical trial. You can usually find this contact information in the trial's description. If you decide to join a clinical trial, let your oncologist know. They may want to talk to the study team to coordinate your care and ensure the trial is safe for you.

"Finding clinical trials is not easy, especially for people who are not familiar with scientific websites and vocabulary. Relying on suggestions from their sarcoma specialist may need to be the first step but it is still important to advocate for oneself as all Oncologists may not be up to date on all trials happening!" *Traci Hurley, M.D. (Pediatrician / CS Caregiver); Phoenix, AZ*

"In 2011, my doctor gave me 3 years to live. Since I literally had nothing to lose, I signed up for my first clinical drug trial. And I've never looked back. I've been through a variety of trials. It's now 2024. The technology and medicine have only gotten better. I'm now 10 years and counting beyond when I was supposed to die. Clinical trials made it happen. It worked for me. There is a reasonable chance it will work for you too. Please, for your sake, ask your oncologist about it! "
Mike Snyder, Chondrosarcoma Warrior, Author: No Treatment Options Left; Albuquerque, NM

"I was a part of a double blinded clinical trial but unfortunately, it did not work for me. My original tumor was in my spine, and I understand people with original spinal tumors are no longer accepted in this study so maybe my participation made a difference. I will do anything to further the research for a cure someday! " *Lynette Veelman, Chondrosarcoma Warrior, Anchorage, AK*

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14 Resources

For more information:

- NIMH's clinical trials webpage
- MedlinePlus (National Library of Medicine) https://medlineplus.gov

(en español) https://medlineplus.gov/spanish

- www.clinicaltrials.gov

(en español) https://clinicaltrials.gov/spanish

- PubMed® database https://www.ncbi.nlm.nih.gov/pubmed
- Clinical Research Trials and You: Questions and Answers

https://www.nimh.nih.gov/health/publications/clinical-research-trials-and-you-questions-and-answers

- The Basics | National Institutes of Health (NIH)

https://www.nih.gov/health-information/nih-clinical-research-trials-you/basics

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