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Clinical Trials: Questions and Answers

What is a clinical trial?

- Clinical trials are research studies that answer scientific questions and try to find better ways to prevent, screen for, diagnose, or treat a disease.
- Taking part in cancer clinical trials allows an opportunity to contribute to knowledge of, and progress against, cancer.
- Participants receive up-to-date care from experts.
- All clinical trials have guidelines about participation.
- Using inclusion/exclusion criteria is an important principle of medical research that helps to produce reliable results.
- Allowing someone to participate in a clinical trial is called inclusion criteria and those that
- Disallowing someone from participating is 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.
- It is important to note that inclusion and exclusion criteria are not used to reject people personally.
- The criteria are used to identify appropriate participants and keep them safe.

What are the types of clinical trials?

Prevention trials test new approaches eg. medications, vitamins, or other supplements, that doctors believe may lower the risk of developing a certain type of cancer.
Most prevention trials are conducted with healthy people who have not had cancer. Some trials are conducted with people who have had cancer and want to prevent recurrence, or reduce the chance of developing a new type of cancer.

Screening trials study ways to detect cancer earlier. They are often conducted to determine whether finding cancer before it causes symptoms decreases the chance of dying from the disease. These trials involve people who do not have any symptoms of cancer.

Diagnostic trials study tests or procedures that could be used to identify cancer more accurately. Diagnostic trials usually include people who have signs or symptoms of cancer.

Treatment trials are conducted with people who have cancer. They are designed to answer specific questions about, and evaluate the effectiveness of, a new treatment or a new way of using a standard treatment. These trials test many types of treatments, such as new drugs, vaccines, new approaches to surgery or radiation therapy, or new combinations of treatments.

Quality-of-life (also called **supportive care**) **trials** explore ways to improve the comfort and quality of life of cancer patients and cancer survivors. These trials may study ways to help people who are experiencing nausea, vomiting, sleep disorders, depression, or other effects from cancer or its treatment.

Genetics studies are sometimes part of another cancer clinical trial. The genetics component of the trial may focus on how genetic makeup can affect detection, diagnosis, or response to cancer treatment.

Population- and family-based genetic research studies In these studies, researchers look at tissue or blood samples, generally from families or large groups of people, to find genetic changes that are associated with cancer. People who participate in genetics studies may or may not have cancer, depending on the study. The goal of these studies is to help understand the role of genes in the development of cancer

What should people consider before participating in a trial?

You should know as much as possible about the clinical trial and feel comfortable asking the members of the treatment team questions about it, the care expected while involved in a trial, and the cost of the trial.
The following questions might be helpful for you to discuss with the treatment team.

The Study

- What is the purpose of the study?
- Why do the researchers think the approach being tested may be effective?
- Has it been tested before?

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Clinical Trials: Questions and Answers cont

Clinical Trials: Q & A cont.

- Who is sponsoring the study?
- Who has reviewed and approved the study?
- What are the medical credentials and experience of the researchers and other study personnel?
- How are the study results and safety of participants being monitored?
- How long will the study last?
- How will the results be shared?

Possible Risks and Benefits

- What are the possible short-term benefits?
- What are the possible long-term benefits?
- What are the short-term risks, such as side effects?
- What are the possible long-term risks?
- What other treatment options are available?
- How do the possible risks and benefits of the trial compare with those of other options?

Participation and Care

- What kinds of treatment, medical tests, or procedures will the participants have during the study?
- How often will they receive the treatments, tests, or procedures?
- Will treatments, tests, or procedures be painful? If so, how can the pain be controlled?
- How do the tests in the study compare with what people might receive outside the study?
- Will participants be able to take their regular medications while in the clinical trial?
- Where will the participants receive their medical care? Will they be in a hospital? If so, for how long?
- Who will be in charge of the participants care? Will they be able to see their own doctors?
- How long will participants need to stay in the study
- Will there be follow-up visits after the study?

Personal Issues

- How could being in the study affect the participants daily lives?
- What support is available for participants and their families?
- Can potential participants talk with people already enrolled in the study?

Cost Issues

- Will participants have to pay for any treatment, tests, Or other charges? If so, what will the approximate charges be?
- Will I be reimbursed for other expenses? Eg. Travelling for review appointments

Why participate in a clinical trial?

Participants in clinical trials can gain access to new research treatments before they are widely available.

How are participants protected?

Research with people is conducted according to strict scientific and ethical principles.

Every clinical trial has a protocol, or action plan. The plan describes what will be done in the study, how it will be conducted, and why each part of the study is necessary. The same protocol is used by every doctor or research centre taking part in the trial.

All clinical trials that are federally funded or that evaluate a new drug or medical device subject must be reviewed and approved by an Ethics Review Board . The Review Board, which includes doctors, researchers, community leaders, and other members of the community, reviews the protocol to make sure the study is conducted fairly and participants are not likely to be harmed. They also decides how often to review the trial once it has begun.

Based on this information, a decision is reached on whether the clinical trial should continue as initially planned and, if not, what changes should be made. A Review Board can stop a clinical trial if the researcher is not following the protocol or if the trial appears to be causing unexpected harm to the participants. They can also stop a clinical trial if there is clear evidence that the new intervention is effective, in order to make it widely available.

What are eligibility criteria, and why are they important?

Each study's protocol has guidelines for who can or cannot participate in the study. These guidelines, called eligibility criteria, describe characteristics that must be shared by all participants. The criteria differ from study to study.

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Eligibility criteria cont.

They may include age, gender, medical history, and current health status. Eligibility criteria for treatment studies often require that patients have a particular type and stage of cancer.

Enrolling participants with similar characteristics helps to ensure that the results of the trial will be due to what is under study and not other factors. In this way, eligibility criteria help researchers achieve accurate and meaningful results. These criteria also minimize the risk of a persons condition becoming worse by participating in the study.

What is informed consent?

It is a process by which people learn the important facts about a clinical trial to help them decide whether to participate. This information includes details about what is involved, such as the purpose of the study, the tests and other procedures used in the study, and the possible risks and benefits. In addition to talking with the doctor or nurse, people receive a written consent form explaining the study.

People who agree to take part in the study are asked to sign the informed consent form. However, signing the form does not mean people must stay in the study. People can leave the study at any time either before the study starts or at any time during the study or the follow-up period.

The informed consent process continues throughout the study. If new benefits, risks, or side effects are discovered during the study, the researchers must inform the participants.

They may be asked to sign new consent forms if they want to stay in the study.

Where do clinical trials take place?

They place in doctors offices, cancer centres, other medical centres, community hospitals and clinics in cities and towns across the World. Clinical trials may include participants at one or two highly specialized centres, or they may involve hundreds of locations at the same time.

How are clinical trials conducted?

They are usually conducted in a series of steps, called phases. Treatment clinical trials are always assigned a phase. However, screening, prevention, diagnostic, and quality-of-life studies do not always have a phase. Genetics clinical trials generally do not have a phase.

Phase I

Are the first step in testing a new approach in people. In these studies, researchers evaluate what dose is safe, how a new agent should be given (by mouth, injected into a vein, or injected into the muscle), and how often. Researchers watch closely for any harmful side effects. **Phase I** trials usually enrol a small number of patients and take place at only a few locations. The dose of the new therapy or technique is increased a little at a time. The highest dose with an acceptable level of side effects is determined to be appropriate for further testing.

Phase II

Study the safety and effectiveness of an agent or intervention, and evaluate how it affects the human body. **Phase II** studies usually focus on a particular type of cancer, and include fewer than 100 patients.

Phase III

Compare a new agent or intervention (or new use of a standard one) with the current standard therapy. Participants are randomly assigned to the standard group or the new group, usually by computer.

This method, called randomization, helps to avoid bias and ensures that human choices or other factors do not affect the studies results. In most cases, studies move into phase III testing only after they have shown promise in phases I and II. **Phase III** trials often include large numbers of people across the country.

Phase IV

Are conducted to further evaluate the long-term safety and effectiveness of a treatment. They usually take place after the treatment has been approved for standard use. Several hundred to several thousand people may take part in a phase IV study. These studies are less common than phase I, II, or III trials. People who participate in a clinical trial work with a research team.

What are some of the benefits of taking part in a clinical trial?

- Participants have access to promising new approaches that are often not available outside the clinical trial setting.

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The benefits of taking part in a clinical trial cont.

- The approach being studied may be more effective than the standard approach.
- Participants receive regular and careful medical attention from a research team that includes doctors and other health professionals.
- Participants may be the first to benefit from the new method under study.
- Results from the study may help others in the future.

What are some of the possible risks associated with taking part in a clinical trial?

Possible risks include the following:

- New drugs or procedures under study are not always better than the standard care to which they are being compared.
- New treatments may have side effects or risks that doctors do not expect or that are worse than those resulting from standard care.
- Participants in randomized trials will not be able to choose the approach they receive.
- Health insurance and managed care providers may not cover all patient care costs in a study.
- Participants may be required to make more visits to the doctor than they would if they were not in the clinical trial.

Who pays for the patient care costs associated with a clinical trial?

Trials are funded by a variety of organizations or individuals such as doctors, medical institutions, foundations, voluntary groups, and pharmaceutical companies.

Trials can take place in a variety of locations, such as hospitals, universities, doctors' offices, or community clinics.

Health insurance and managed care providers often do not cover the patient care costs associated with a clinical trial. What they cover varies by health plan and by study.

What is a protocol?

It is a study plan on which all clinical trials are based. The plan is carefully designed to safeguard the health of the participants as well as answer specific research questions. A protocol describes what types of people may participate in the trial; the schedule of tests, procedures, medications, and dosages; and the length of the study. While in a clinical trial, participants following a protocol are seen regularly by the research staff to monitor their health and to determine the safety and effectiveness of their treatment.

What is a placebo?

It is an inactive capsule, tablet, liquid, or powder that has no treatment value. In clinical trials, experimental treatments are often compared with placebos to assess the treatment's effectiveness. In these studies, the participants in the control group will receive a placebo instead of an active drug or treatment.

What is a control or control group?

A control is the standard by which experimental observations are evaluated. In many clinical trials, one group of patients will be given an experimental drug or treatment, while the control group is given either a standard treatment for the illness or a placebo.

What is an "expanded access" protocol?

Sometimes, patients do not qualify for carefully-controlled trials because of other health problems, age, or other factors. For patients who may benefit from the drug use but don't qualify for the trials, government regulations may enable manufacturers of investigational new drugs to provide for "expanded access" use of the drug.

The primary intent of a treatment protocol is to provide for access to promising new drugs for people with a life-threatening or serious disease for which there is no good alternative treatment.

Expanded access protocols can be undertaken only if clinical investigators are actively studying the new treatment in well-controlled studies, or all studies have been completed. There must be evidence that the drug may be an effective treatment in patients like those to be treated under the protocol. The drug cannot expose patients to unreasonable risks given the severity of the disease to be treated.

Expanded access protocols are generally managed by the manufacturer, with the investigational treatment administered by researchers or doctors in office-based practice.

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What happens during a clinical trial?

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.

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

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.

In addition, 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 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 both immediate and long-term side effects.

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 controlled protocol, a study plan that 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 secret and will not be mentioned in these reports.

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.

What happens when a clinical trial is over?

After a clinical trial is completed, the researchers look carefully at the data collected during the trial before making decisions about the meaning of the findings and further testing. After a phase I or II trial, the researchers decide whether to move on to the next phase, or stop testing the agent or intervention because it was not safe or effective. When a phase III trial is completed, the researchers look at the data and decide whether the results have medical importance.

The results of clinical trials are often published in peer-reviewed, scientific journals. Peer review is a process by which experts review the report before it is published to make sure the analysis and conclusions are sound. If the results are particularly important, they may be featured by the media and discussed at scientific meetings and by patient advocacy groups before they are published. Once a new approach has been proven safe and effective in a clinical trial, it may become standard practice. (Standard practice is a currently accepted and widely used approach.)

Worldwide Clinical Trial Listings

- **http://www.clinicaltrialssearch.org/148-cancer-clinical-trials.html**
- USA **http://www.clinicaltrials.gov**
- USA **http://www.virtualtrials.com/**

Choosing to participate in a clinical trial is an important personal decision.